

# RARE CONVERSATIONS

## A Vision for Rare Disease Policy

2023 Report



Rare Conversations was convened by Alexion, AstraZeneca Rare Disease, in collaboration with the Rare Conversations Steering Committee. We thank them for their input and participation in the program.

Opinions, conclusions, and recommendations expressed or implied in this report aim to accurately capture and summarize the discussions that took place on December 6, 2023 at Rare Conversations, and do not necessarily represent the official views of Alexion or Rare Conversations Steering Committee members.

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# Convening the Discussion

In 1983, Congress passed the Orphan Drug Act (ODA) in service to one central goal – to stimulate the development of new treatments for rare diseases. Each new rare disease therapy that has been approved since the ODA became law is a compelling reminder of the power of policy to drive progress for patients and caregivers.

Continued innovation depends on sound policymaking. There are 30 million Americans living with a rare disease<sup>i</sup> and many of those are children.

It has been said that rare disease patients are among the most marginalized Americans, regardless of race, ethnicity, socioeconomic status, genetics, or ZIP code.<sup>ii</sup> Most people living with a rare disease endure an arduous road of misdiagnoses or dismissed symptoms – a journey that lasts an average of 6 years and involves an average of 17 specialists.<sup>iii</sup> Approximately 95% of rare diseases<sup>iv</sup> today still have no U.S. Food and Drug Administration (FDA)-approved treatment options, despite the surge of new therapies that have emerged since the ODA. Even for those rare diseases with available treatments, patients often encounter barriers to accessing them.

These challenges make it vital to support policies that fuel the next 40 years of innovation. We convened Rare Conversations on December 6, 2023, in Washington, DC, to create a forum for exploring the policy issues that affect the rare disease community. We brought together diverse voices – patients, caregivers, policymakers, researchers, clinicians, and regulators – to inform the conversation and identify actions we can take to effect change.

It's essential that we hear what was said. Each of us has a role to play in driving progress that is urgently needed. We hope these perspectives and insights inspire each of us to take action and create a better future for people living with rare diseases.

Rare Conversations Steering Committee Members:



# Introduction

At Rare Conversations, common themes emerged, particularly concerns about protecting the progress fostered by the ODA, addressing the harms of the Inflation Reduction Act (IRA), and ensuring patient access to transformative cell and gene therapies. Participants recognized that while there may be different approaches to serving the needs of the community, it's essential for rare disease advocates to come together to shape sound health policies. This report summarizes stakeholder perspectives on policy priorities and barriers, considers areas of agreement, and highlights what the rare disease community can do to meet the needs of patients and caregivers.



“There’s an African proverb that says ‘If you want to go fast, go it alone. If you want to go far, go it together.’ Today’s conversations will help us travel together toward a better tomorrow for all the rare disease patients and the families we serve.”

**TAMAR THOMPSON**

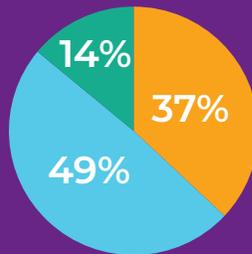
Vice President, Head of Corporate Affairs,  
Alexion, AstraZeneca Rare Disease

## Raising our voices:

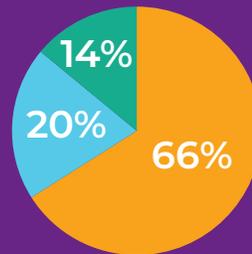
*Do the policies of government agencies adequately address the needs of the rare disease community?*

Conference participants said:

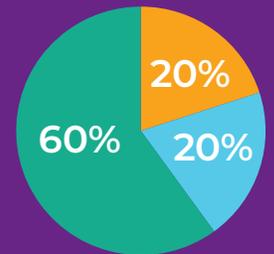
■ Agree   
 ■ Disagree   
 ■ Don't know



Federal



FDA



State Legislators

# Hear Our Voices: Advocating for Our Community

The power of the patient voice should never be underestimated. Advocates like Dorothea Lantz, Director of Community Engagement at the Prader-Willi Syndrome Association, illustrate why. When her son, Hunter, was diagnosed with Prader-Willi Syndrome (PWS), his health care team outlined all the limitations he would face. She quickly turned those limitations into possibilities by advocating for her son and engaging him in rigorous therapy to improve his life.

As she became more deeply involved in the advocacy community, Lantz soon saw that she and other families weren't alone. They were part of a much larger rare disease community. She worked with other parents to interest biotech companies in developing treatments for this rare disease. While their work resulted in clinical trials of potentially promising medicines, Lantz began to see how policies were eroding incentives for biotech companies to take on the high-risk effort. She called out persistent legislative attacks on the orphan drug tax credit, a vital part of the ODA.

Lantz illustrated why policies that inhibit investment in rare diseases are so shortsighted. Progress begets progress, and rare disease treatments that can benefit a small number of people can translate into new treatments for other, more common conditions. She noted that medicines under development for PWS may also prove to be effective for broader clinical use.

Lantz reminds us that collectively, people living with a rare disease are not rare. About 30 million Americans live with a rare disease and millions more caregivers volunteer their time to help them cope. It's vital to raise our voices to ensure that policies reflect the needs of patients and help optimize the process of receiving a diagnosis and accessing innovative treatments.

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"I hope that policymakers are hearing us. The rare disease community cannot afford to stand by and watch as those who believe they're unaffected by rare diseases continue to erode decades of progress for our families. Every time Congress enacts new health care legislation, we fear that government agencies will misinterpret Congressional intention. When that happens, people like my son suffer."

**DOROTHEA LANTZ**

Director of Community Engagement,  
Prader-Willi Syndrome Association

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## Policy for Progress: Understanding the Successes of the Orphan Drug Act

Over the last 40 years, the ODA has generated nothing short of a transformation in the rare disease landscape. Before the law's passage, there were fewer than 40 drugs approved to treat rare diseases. Driven by the work of advocates, the ODA was passed by Congress with strong bipartisan support. The ODA combined multiple approaches to incentivize companies taking on high-risk efforts to develop rare disease treatments: defining rare diseases as those affecting fewer than 200,000 people, creating a tax credit, protecting market exclusivity, and exempting companies from user fees.<sup>v</sup> Today, more than 880 drugs capable of treating over 1,200 indications have been approved by the FDA.<sup>vi,vii</sup>

Despite the ODA's incentives and the corresponding increase in investment, developing new treatments remains challenging and risky for companies working to innovate for people living with rare

diseases. Fewer than 10% of the thousands of investigational drugs being researched ever obtain FDA approval for use in patients.<sup>viii</sup>

Instead of protecting the fragile balance between risk and incentives, policymakers have begun to chip away at existing orphan drug incentives, putting an already vulnerable community at risk. In 2017, lawmakers in the U.S. House of Representatives passed legislation to fully repeal the tax incentive, which credited companies 50% of the costs of clinical trials.<sup>ix</sup> It was only through the unwavering advocacy of the rare disease community that a 25% tax credit remained intact.<sup>x</sup>

Patient-focused policies, like strengthening the Orphan Drug Tax Credit, are critical to addressing the risk of developing treatments for rare diseases.

## Unintended Consequences: Recognizing the Harms of the Inflation Reduction Act

Throughout Rare Conversations, participants raised concerns over the IRA and provisions that further erode incentives for rare disease drug development.

Under the IRA, certain orphan drugs are excluded from the Medicare Drug Price Negotiation Program. But the exclusion only applies to orphan drugs that treat a single indication, or specific use, not those used for multiple indications. Currently, roughly one quarter of all orphan drugs approved in the last two decades have a single indication.<sup>xi</sup> Researching additional orphan indications for existing rare disease treatments is an important aspect of rare disease research, accelerating scientific progress and allowing innovative therapies to reach more patients.

Biopharmaceutical manufacturers have expressed concerns that the narrow Orphan Drug Exclusion is slowing the development of new treatments for rare diseases and discourages biopharmaceutical companies from bringing safe and effective therapies or potential cures to patients.<sup>xii</sup>

Another concern with the IRA is that it discourages the development of therapies that come in pill form. Scott Weintraub, Senior Vice President, United States Business, Alexion, AstraZeneca Rare Disease noted studies that have demonstrated the disincentives created by the IRA for developing small molecule drugs, which are typically administered as a pill or capsule, and incentivizes the development of large molecule drugs, which must be administered intravenously or through infusion.<sup>xiii</sup>



Scott Weintraub, Senior Vice President, United States Business, Alexion, AstraZeneca Rare Disease (left), discusses legislative challenges with Steve Usdin, Washington Editor, *BioCentury*.

“The dynamics of rare disease research – finding patients, knowing what to measure in absence of natural history studies, aligning with regulatory agencies on meaningful endpoints – is all very difficult. That doesn't change the fact that there's a high unmet medical need, so we need policies in place to drive progress.”

### SCOTT WEINTRAUB

Senior Vice President, United States Business,  
Alexion, AstraZeneca Rare Disease

# Views from Capitol Hill

## Legislators – On the Front Lines of Policy



“My son has big dreams and we’re working to help him fulfill them. Everyone should have access to the treatments they need to live life to the fullest.”

### CATHY MCMORRIS RODGERS

United States Representative (R-WA)

U.S. Representatives Cathy McMorris Rodgers (R-WA) and Wiley Nickel (D-NC) joined Rare Conversations and shared their perspectives on how Congress can advance research and development of new treatments for people living with rare diseases.

Each shared personal stories related to family members with serious health conditions, and these experiences have shaped their commitment to the rare disease community. McMorris Rodgers' oldest child was born with Down Syndrome, giving her a keen understanding of raising a child with special health care needs. Nickel was only 18 when he lost his father to lung cancer, and he saw firsthand the pain of losing a loved one who had no more treatment options.

Both recognize the power of policy to deliver better health care to their constituents.

McMorris Rodgers highlighted several issues that pose a threat to creating new orphan drugs, including the use of quality-adjusted life years (QALYs), a measure that some payer organizations use to determine the value of treatments and their coverage decisions. If passed, McMorris Rodgers' Protecting Health Care for All Patients Act of 2023<sup>xiiv</sup> would ensure the U.S. government does not use QALYs to decide the worth of a person's life. She also outlined the importance of modernizing the FDA's Accelerated Approval Program, which fosters earlier approval of drugs to fill an unmet need.

Additionally, both Representatives discussed the importance of encouraging continued rare disease drug discovery and development by addressing concerns about the IRA's drug pricing provisions. Legislators are collaborating across the aisle to restore incentives for research, development, and access to rare disease treatments. Nickel spoke about his introduction of the Optimizing Research Progress Hope and New Cures (ORPHAN Cures) Act.<sup>xv</sup> To support changes to the IRA, he encouraged the community to take the time to educate lawmakers, illustrate the importance of incentivizing cures by sharing front-line perspectives, thank representatives when they champion priority issues (even when there's disagreement on other issues), and support bipartisan efforts.



Wiley Nickel, United States Representative (D-NC) engages in a fireside chat with Katie Jones, Senior Director of Federal Affairs, Alexion, AstraZeneca Rare Disease.

“Every person here has members of Congress who represent you. They’re your voice. Do everything you can to meet with them and educate them. They want to hear from you.”

### WILEY NICKEL

United States Representative (D-NC)

## Legislative Staff – Working Behind the Scenes for the Community

The detailed, painstaking work of crafting legislation is driven by legislators' staff on Capitol Hill, evidenced by the two experienced legislative directors who joined Rare Conversations for a discussion moderated by Josh Trent, Managing Principal, Leavitt Partners. Dominique Yelinski, then Deputy Chief of Staff and Legislative Director for Representative Kevin Hern (R-OK), and Corey Malmgren, former Deputy Legislative Director and Health Counsel for Senator Bill Nelson (D-FL) and Legislative Counsel for Representative Sandy Levin (D-MI), spoke from both sides of the aisle.

They discussed the importance of protecting the ODA and addressing concerns about the IRA's chilling effects on the development of rare disease treatments.

While proponents of the IRA may see the need for "improvements" and critics of the law may see the need for "fixes," both Yelinski and Malmgren agreed that bipartisan collaboration will be essential to success. It won't be an easy road, however. With 2024 a presidential election year, they noted that the near-term environment will be more fractured and more difficult for both parties to work together to benefit people living with rare diseases.

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"Members of Congress no longer have a bipartisan forum to talk about important issues. It's incumbent on all of us to force that interaction - and force that friendship - that's really eroded in the past 10 years or so. Building those relationships will lead to strong support of our priorities."

### COREY MALMGREN

Former Deputy Legislative Director and Health Counsel for Senator Bill Nelson (D-FL)

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How can the rare disease community help overcome these obstacles? Malmgren and Yelinski offered recommendations for success:

- **Be ready to educate** – Future legislative action depends on ensuring legislators and staff understand the issues and the science behind them. Whether it's explaining what a biologic or a small molecule is, or outlining how patients benefit from multiple indications for treatments, it's essential to convey why they need to act in terms they understand.
- **Include community voices** – Yelinski and Malmgren noted the power of hearing from patients, families, and researchers, and illustrating the impact of policy barriers to research and development.

- **Engage the right people** – Yelinski and Malmgren encouraged advocates to work their way through Congressional offices, meeting first with health staff, then chiefs of staff and ultimately, members of Congress themselves. They said advocates should first identify members on the most relevant committees and those who have been outspoken on rare disease issues. It's also important to engage with other influential members who will vote on the legislation.
- **Create a forum** – Most important, both experts noted that political polarization means that members seeking solutions need to have a place to come together. Stakeholders can help foster the discussion and relationships necessary to drive success.

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"[Addressing the IRA] is such an important issue, and it would be a horrible waste to make it a partisan issue. People's lives are at stake."

### DOMINIQUE YELINSKI

Former Deputy Chief of Staff and Legislative Director for Representative Kevin Hern (R-OK)



(Left to right) Josh Trent, Managing Principal at Leavitt Partners, Corey Malmgren, former Deputy Legislative Director and Health Counsel for Senator Bill Nelson (D-FL), and Dominique Yelinski, former Deputy Chief of Staff and Legislative Director for Representative Kevin Hern (R-OK), share best practices for working with members of Congress.

# Views from the Community: Policy Opportunities and Challenges Around Cell and Gene Therapies

The benefits of new cell and gene therapies require policies that ensure appropriate patient access. Christina Hochul, Senior Director, Head of Strategic Alliance Development at Alexion, AstraZeneca Rare Disease, moderated a discussion on key challenges and opportunities to bring these life-changing therapies to people living with rare diseases. The panel included three experts: Tim Hunt, Chief Executive Officer of the Alliance for Regenerative Medicine; Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement of the EveryLife Foundation for Rare Diseases; and Jason Resendez, President and Chief Executive Officer of the National Alliance for Caregiving.

Hunt set the stage by illustrating how dramatically the field has changed in recent years. The potential for gene therapy in rare diseases was first described in a paper published in 1972, and it took more than four decades for the first therapy to be approved in 2017.<sup>xvi</sup> Between 2017 and 2021, five new treatments were approved by the FDA. In 2023 alone, an additional five treatments were approved.

Cell and gene therapies can transform or even cure people living with a rare disorder, often with a single administration. Though costly, they can significantly reduce spending on chronic and acute care currently paid for over a person's lifetime. However, that requires a large upfront investment in exchange for years, decades, or a lifetime of benefits.

Policymakers must move quickly to match the rapid pace of scientific innovation with policies that ensure timely, equitable, and appropriate access to newly approved cell and gene therapies. The panelists outlined challenges and opportunities in two key areas: regulatory and access.

## Regulatory

The panel recognized the FDA's pivotal role in changing patients' lives, especially by addressing current needs and anticipating what lies ahead. Yet, they noted that the FDA does not currently have the infrastructure, people, or bandwidth to manage the increasing number and complexity of potential approvals of cell and gene therapies. Under the leadership of Peter Marks, MD, PhD, Director, Center for Biologics Evaluation and Research, the FDA is building additional infrastructure and more responsive review policies. Panelists suggested that the rare disease community should contribute to, and advocate for, FDA's essential policies and budget requests to help the agency meet its goals.

One area that is rarely assessed in clinical trials and drug development is the caregiver experience. There are 53 million unpaid family caregivers who are an essential extension of the care team.<sup>xvii</sup> Resendez noted the importance of engaging with the FDA to define how to report and measure caregivers' contributions, to ensure the full impact of treatments is well understood.

The panel also highlighted the importance of improving data collection about the full patient and caregiver experience. Data on costs and other aspects of patient care may not inform regulatory decisions but can impact access-related decisions, such as insurance coverage. Improved data collection will require systemic changes, including medical coding. Among some 10,000 rare diseases, there are fewer than 600 related International Classification of Diseases (ICD) codes, which are essential for data collection and reimbursement.<sup>xviii</sup>

“We spend a lot of time talking about how much it’s going to cost to invest in innovative therapies – for good reason. But we’re already paying for the cost of rare diseases in a lot of other different ways. The lion’s share of these costs is already shouldered by families and society. Unless we bring that data to them, policymakers won’t see it.”

### ANNIE KENNEDY

Chief of Policy, Advocacy, and Patient Engagement, EveryLife Foundation for Rare Diseases

*What are the 3 words that come to mind when you think of cell and gene therapies?*



Alleviating caregiver hardship

Jason Resendez



Medicaid, access, hope

Tim Hunt



Cautious optimism and gratitude

Annie Kennedy

# Rare Conversations



The discussion on cell and gene therapies featured (left to right) moderator Christina Hochul, Senior Director, Head of Strategic Alliance Development at Alexion, AstraZeneca Rare Disease, Jason Resendez, President and Chief Executive Officer of the National Alliance for Caregiving, Tim Hunt, Chief Executive Officer of the Alliance for Regenerative Medicine, and Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement of the EveryLife Foundation for Rare Diseases.

## Access

Kennedy noted that the costs of cell and gene therapies are the focus of policy discussions because they are easy to see. It's vital for policymakers to understand the burden of what is already spent by the health care system and families, such as costs related to delayed diagnosis, chronic care, and caregiving.

The panel also discussed needed changes to Medicaid, which provides health coverage for millions of low-income and medically eligible Americans. Medicaid is administered by the states and policies can differ significantly between them. Costs and spending are typically evaluated in one-year budget cycles. That creates unique challenges when the costs of cell and gene therapies are incurred upfront but their *benefits* – including reduced health care spending, lower caregiver burden, and increased productivity – accrue over time. In addition, if patients move, the state that paid for the treatment may not realize the long-term benefits of paying for treatment today. Medicaid patients can encounter barriers when seeking care out-of-state – a common situation due to the limited number of treatment centers. Effective policies will require exploring novel solutions to these pressing challenges, such as pooling aspects of state Medicaid programs and developing outcomes-based agreements.

Education and awareness are also important components of access. Panelists agreed that patients must have the opportunity

to learn about new therapies, how they work, and what must be done to use them effectively. Health care providers may also need training to effectively educate patients about how to use treatments appropriately. Resendez advocated for including caregivers in training and education to ensure they're equipped to support their loved ones. More than half of caregivers report performing medical tasks,<sup>ix</sup> often without training.

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“Society and payers, including Medicaid, can afford the costs of cell and gene therapies. We have good data that, by 2030, total projected costs of these therapies will be about 1.3% of estimated total prescription drug spending. This is important context for people who see the costs of these therapies and worry we're going to go broke.”

### TIM HUNT

Chief Executive Officer,  
Alliance for Regenerative Medicine

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# Delivering for Patients: Regulatory Reform for Rare Disease Therapies

Helping safe and effective new treatments reach people living with rare diseases is the goal of advocates, researchers, clinicians, industry, and the FDA. During Rare Conversations, a group of experts came together to discuss regulatory reforms that could help accelerate the process. The conversation was moderated by Heidi Ross, Vice President of Policy and Regulatory Affairs of the National Organization for Rare Disorders, and included Hilary Marston, Chief Medical Officer of the FDA; Eliza Lo Chin, Executive Director of the American Medical Women's Association; and Lee Fleisher, a Senior Advisor to FasterCures. The discussion explored several key topics.

Marston described the FDA's work to encourage scientific breakthroughs by engaging with industry, patients, and foundations to support a more efficient product development process. She highlighted several FDA initiatives aimed at addressing gaps across the spectrum of orphan drug development, including the Support for clinical Trials Advancing Rare disease Therapeutics (START) Pilot Program, the Accelerating Rare disease Cures (ARC) Program, and the voluntary Total Product Life Cycle (TPLC) Advisory Program (TAP) Pilot. Marston encouraged attendees to share their feedback on these programs to ensure they are working for the community.

“At the FDA, we’ve been working for four decades to ensure safe and effective treatments reach patients with rare diseases. We’ve made important progress and there are now treatments for 1,200 indications in rare disease. But we are nowhere near done and we have quite a bit more work to do.”



**HILARY MARSTON**  
Chief Medical Officer, FDA

A priority for the FDA and advocacy organizations is promoting diversity in clinical trials. While the Food and Drug Omnibus Reform Act (FDORA) of 2022<sup>xx</sup> gave the FDA more authority to ensure clinical trials reflect the diversity of patients, determining what representative patient enrollment looks like in a particular disease is a challenge. FDA offers grants to support natural history studies that yield better understanding of patient demographics. While investing in natural history studies is essential to achieving diverse clinical trials, success will require other cross-cutting approaches, including more provider and patient education.

The FDA also works to improve the speed and flexibility of clinical trials. A survey conducted by NORD in 2019 showed that almost



Lee Fleisher, Senior Advisor, FasterCures, discusses regulatory reform with panel moderator Heidi Ross, Vice President of Policy and Regulatory Affairs, National Organization for Rare Disorders, as Eliza Lo Chin, Executive Director, American Medical Women's Association, looks on.

40% of rare disease patients travel more than 60 miles to see their health care provider, and the FDA sees opportunities to alleviate the burden patients face through decentralized clinical trials.<sup>xxi</sup> To better leverage telehealth, it's essential to remove barriers to cross-state care and reimbursement for telehealth visits. Remote patient monitoring, local blood draws for lab work, remote patient consent, and other innovations should be integrated into the health care system.

Another area of promise is accelerating research through real-world evidence. Real-world studies examine data that reflect a patient's health experience in the day-to-day world, using electronic health records, registries, payer and claims data, as well as health apps or wearable devices. The FDA has been working for several years to offer guidance on the conduct of real-world studies and how evidence needs to be presented to inform regulatory review.

Fleisher discussed why it's especially important for the rare disease community to align with the FDA and the National Institutes of Health (NIH) on the surrogate endpoints used in research. He described how the Centers for Medicare and Medicaid Services (CMS) makes coverage decisions about treatments, particularly by Medicare. While some coverage decisions are made on a national level, most are made by 13 Medicare Administrative Coordinators across the country. This creates variability in how treatments are covered by the program, making well-defined and broadly accepted endpoints important to support CMS coverage decisions.

“The mantra in medicine is ‘common things are common and if you hear hoofbeats, look for horses, not zebras.’ But 1 in 10 Americans is suspected of having a rare disease or are diagnosed with a rare disease. That means there are a lot of zebras amongst the horses.”



**HEIDI ROSS**  
Vice President of Policy and Regulatory Affairs,  
National Organization for Rare Disorders

# Forward Together: Next Steps in Advancing Rare Disease Policy

Through open dialogue and engaging discussions, Rare Conversations helped participants form a clear picture of today's policy landscape. While there has been great progress in the last 40 years, action is urgently needed to address new challenges and threats. During the closing panel, Christina Hochul and Michele Oshman, Vice President for External Affairs, Biotechnology Innovation Organization, highlighted the incredible value and diverse perspectives experts brought to Rare Conversations, and encouraged advocates to support four key takeaways:

## 1. Foster an informed policy debate.

- **Educate policymakers:** Advocates should elevate perspectives from patients, caregivers, researchers, and clinicians and recognize policymakers' different learning styles and needs.
- **Enable bipartisan discussion:** Because much-needed platforms don't currently exist on Capitol Hill, rare disease stakeholders should convene meetings, conferences and other forums that help policymakers reach across the aisle.
- **Focus and align on priorities:** The rare disease community can create the greatest impact by identifying areas of agreement and centering them in advocacy efforts.

## 2. Address legislative policies that erode incentives for rare disease therapies.

- **Orphan Drug Tax Credit:** Preserving tax credits for qualified clinical testing expenses will help balance the risk biopharmaceutical companies take on in rare disease research and drug development.
- **The IRA's Orphan Drug Exclusion:** Improving and clarifying this provision will protect rare disease patients by promoting additional designations and indications for new treatments for rare diseases.
- **The FDA's Accelerated Approval Pathway:** Strengthening and fostering broader use of this program will allow approval of more rare disease therapies through the use of surrogate endpoints.

Progress and success in each of these areas will require ongoing conversation, commitment, and collaboration across the entire rare disease community. By coming together, we can break down policy barriers that hinder progress and deliver life-changing innovation to people living with rare diseases. In doing so, we can improve people's health, reduce reliance on health care and other services, lower caregivers' cost burden, and promote value across the entire health care system.

When we all come together, the rare disease universe is a large, strong community with the power to shape policies that affect patients and caregivers. The voices of all people living with rare



Michele Oshman, Vice President for External Affairs, Biotechnology Innovation Organization, summarizes the policy areas of agreement discussed throughout the day's event with Christina Hochul of Alexion, AstraZeneca Rare Disease.

## 3. Promote FDA reforms to expedite approval of new therapies.

- **Ensure capacity:** Advocate for the necessary infrastructure at FDA to review the growing number of cell and gene therapies.
- **Make trials more accessible:** Engage with the FDA to shape agency guidance on developing clinical endpoints that support review and improve the use of telehealth, decentralized trials, and real-world studies, for example.

## 4. Facilitate patient access to cell and gene therapies through sustainable and innovative payment mechanisms.

- **CMS:** Pilot innovative payment models in state Medicaid programs, like value-based payment arrangements or provisions that ensure cost does not inhibit patient access.
- **CMS Innovation Center (CMMI):** Engage in and support CMMI's novel payment approaches, including outcomes-based agreements. Specifically, the Innovation Center has been seeking and collecting public feedback on holistically addressing access and affordability among underserved populations.
- **Capture a holistic picture:** Ensure caregiver experiences are captured in data from the start and reflected in outcomes to inform discussions on costs, coverage, and value.

diseases, caregivers, and advocates matter. We invite you to stay involved in the conversation, share your perspectives and advocate with us, the rare disease community.



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