RDDC

RARE DISEASE DIVERSITY COALITION

# 2025 ACHIEVEMENT REPORT

BLACK WOMEN'S HEALTH IMPERATIVE

# RDDC

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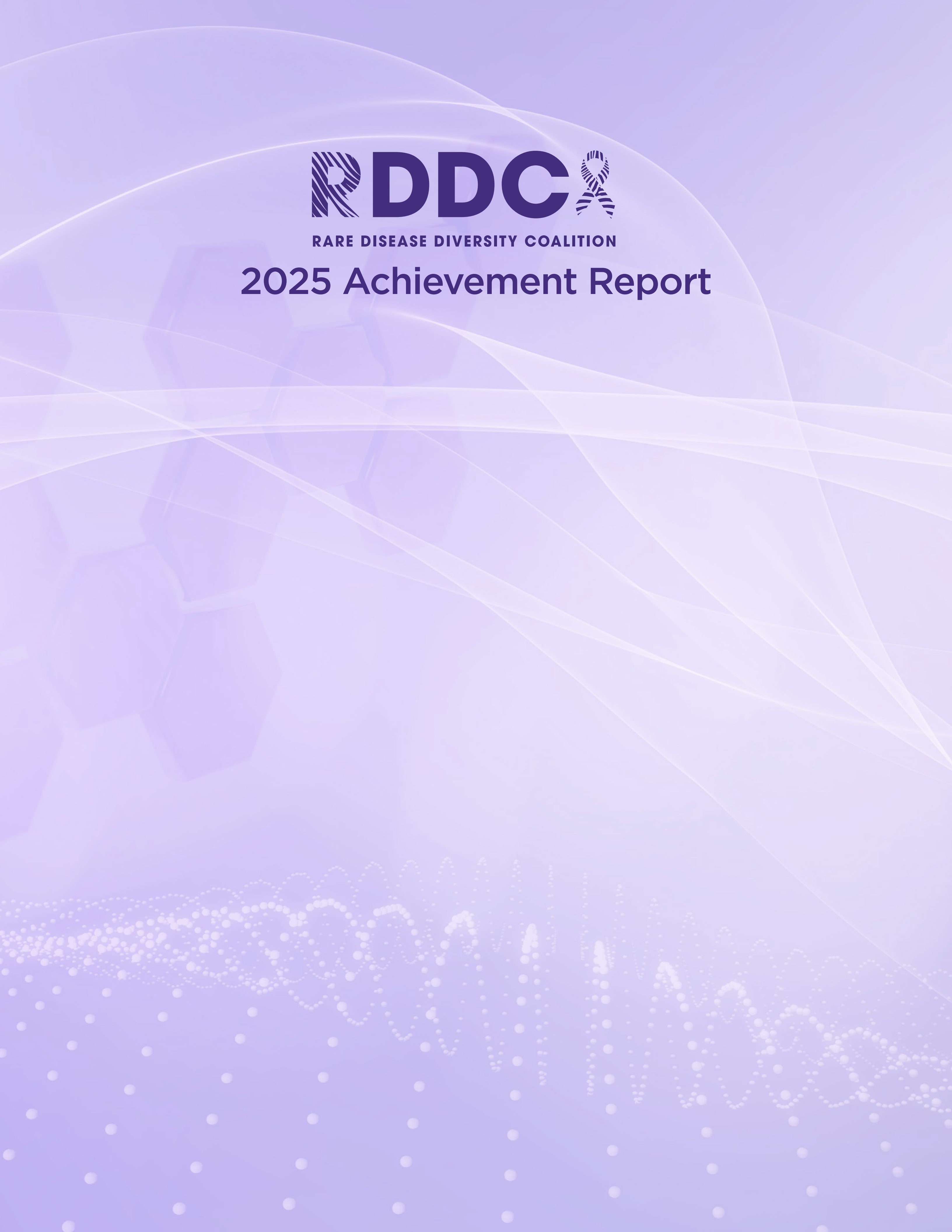
# 2025 Achievement Report



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RDDC

RARE DISEASE DIVERSITY COALITION

# Introduction

The Rare Disease Diversity Coalition (RDDC) brings together individuals and organizations to confront the distinct challenges faced by patients from historically marginalized communities, along with their families and broader support networks.

Though rare diseases may seem uncommon, they affect an estimated 25–30 million people in the United States. With approximately 10,000 known rare diseases, the diversity within this patient population is vast—and so are the disparities that people who have them face.

Patients from underrepresented communities often experience significant barriers in the rare disease space. They are more likely to encounter delays in diagnosis, which can lead to diminished access to effective treatment and care along with poorer health outcomes. Their unique needs and lived experiences are often misunderstood or overlooked by healthcare providers. Additionally, rare diseases that disproportionately affect communities of color frequently receive less research funding, further exacerbating inequities.

As RDDC closes out its fifth year in 2025, our mission remains steadfast – to promote equitable access to diagnosis, treatment, and care for rare disease patients, especially those from underrepresented backgrounds. We are committed to elevating their voices, addressing their specific challenges, and ensuring they are not only heard but truly understood.

At the heart of our work is collaboration. RDDC recognizes that meaningful change requires the insight and partnership of all stakeholders – patients and families who live these realities every day, patient organizations that advocate and organize, clinicians and researchers who deepen medical understanding, private-sector partners who advance innovation and investment, and policymakers who drive systemic change. Together, we can create the momentum needed to transform rare disease care.

RDDC remains unwavering in its commitment to identifying and championing evidence-based solutions that close gaps in access, improve diagnostic timelines, and build a more equitable future for all individuals affected by rare diseases.

## 2025 KEY ACHIEVEMENTS

In 2025, several key achievements have marked significant milestones across RDDC, including:

- *Inequities in the Rare Disease Community: The Voices of Diverse Patients and Caregivers* Report
  - In late August 2024, in partnership with the National Organization for Rare Disorders (NORD), RDDC produced and disseminated the *Inequities in the Rare Disease Community: The Voices of Diverse Patients and Caregivers* Report. Additional report highlights are listed in the Patient-Provider-Caregiver Journey Workgroup section.

- **Co-sponsor of the Fourth Annual Global Genes Rare Health Equity Forum** in September 2024. The theme was, "Together in Equity, Driving Change". Through plenary sessions, networking, and interactive table talks, the event empowered attendees with actionable tools and strategic insights to advocate for a more inclusive rare disease community.[1]

- **Rare Disease Improving Health Equity CME Webinar Series**
  - The Bluff City Medical Society Foundation, in partnership with RDDC, launched a 3-part webinar series titled, *Improving Health Equity: Implications for Rare Disease*. To read more about this work, visit the Patient-Provider-Caregiver Journey Workgroup section.

- **Paroxysmal Nocturnal Hemoglobinuria (PNH) White Paper**
  - In January 2025, RDDC and Links2Equity presented a white paper titled, *Health Equity for People Living with PNH: Creating a Leveled Playing Field*. This paper was made possible through the insights and contributions of patient advocates, healthcare professionals, advocates, and others who have lived experiences with PNH. It was developed to achieve greater equity for patients living with PNH, with recommendations to improve equitable access to care, reduce financial barriers, and enhance provider education. Please share with your PNH communities.
  - **Press Release:** Rare Disease Diversity Coalition Releases Groundbreaking Report to Advance Health Equity for Patients with Ultra-Rare Blood Disorder
    - Visibility Report as of May 27, 2025:
      - Potential audience: 243.7 million
      - Exact matches: 632
      - AP outlet distribution: 889 outlets
      - Release views: 5,012
      - Click-throughs: 242
    - Social Media Toolkit: *Health Equity for People Living with PNH*

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[1] https://globalgenes.org/rare-health-equity-forum/

- **Vasculitis Patient Navigation Fact Sheet**
  - In January 2025, RDDC, in partnership with the Vasculitis Foundation, delivered a 3-page vasculitis fact sheet titled, *Living Well with Vasculitis: Essential Topics to Discuss with Your Provider*. Living with vasculitis requires a proactive approach to managing health. Building a strong relationship with a healthcare team is essential to ensuring that patients receive the best possible care. This fact sheet is designed to help patients navigate important conversations with their providers, empowering them to take an active role in their treatment. It explores key topics to help patients and their healthcare team create a personalized plan that addresses the patient's unique needs and improves the patient's quality of life.

- **RDDC hosted its third annual event, "The Eve of Rare Disease Day Reception".** This intimate event brought together more than 50 attendees, including rare disease patients, caregivers, advocacy organizational leaders, government representatives, and industry professionals.

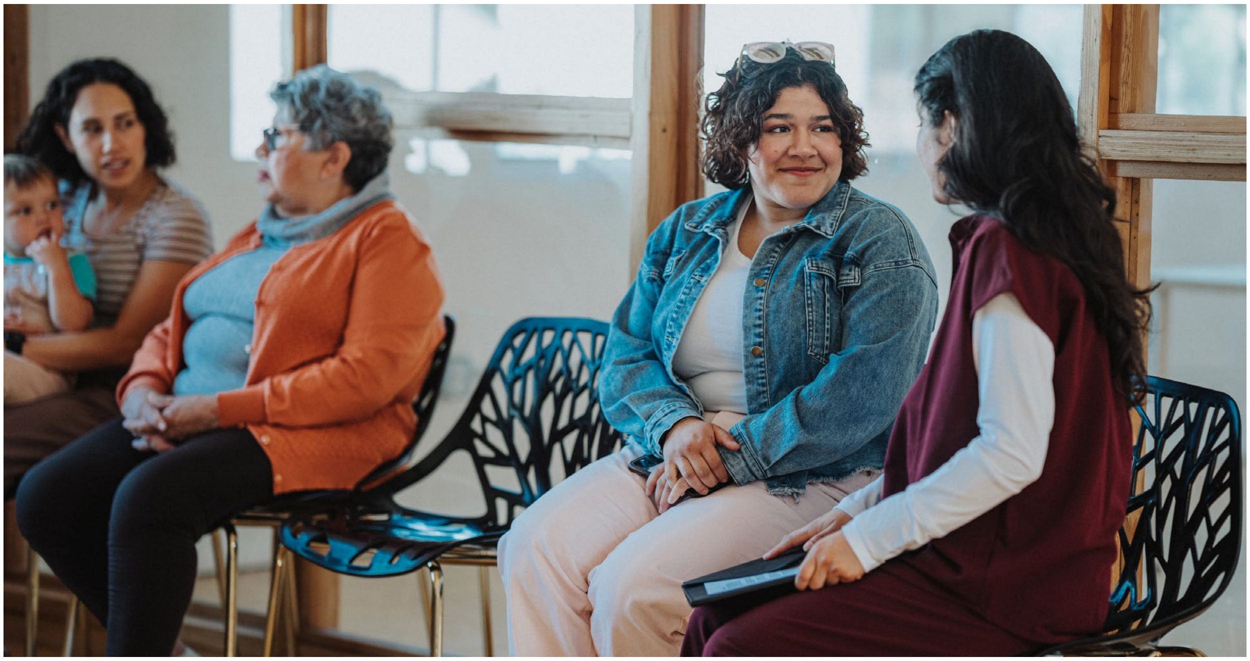
  - The event included the RISE Award ceremony, at which nine rare disease champions were presented with inaugural RISE Awards. RISE stands for Resilient, Impassioned, Strong, and Empowered. The awards highlighted members and organizations of the rare disease community who encompassed the RDDC mission and led the charge for rare disease diversity advocacy:
    - 2025 Patient RISE Award – Elizabeth Kennerly
    - 2025 Provider RISE Award – Dr. LaTonya Washington
    - 2025 Caregiver RISE Award – Dr. Harsha Rajasimha
    - 2025 Patient Advocacy RISE Award – Stephanie Marshall
    - 2025 Industry RISE Award – Novartis
    - 2025 Health Equity RISE Award – National Institutes of Health, National Center for Advancing Translational Sciences
    - 2025 Honoree RISE Award – Rare Diseases Clinical Research Network
    - 2025 Congressional RISE Award – Senator Amy Klobuchar
    - 2025 Congressional RISE Award – Senator Bill Cassidy
  - Press Release: Rare Disease Diversity Coalition to Host 2nd Annual RISE Awards on the Eve of Rare Disease Day
    - Visibility Report as of May 27, 2025:
      - Potential audience: 167.4 million
      - Exact matches: 619
      - AP outlet distribution: 956 outlets
      - Release views: 2,423
      - Click-throughs: 40
  - RDDC Rare Disease Day Social Media Toolkit: RDDC Celebrates Rare Disease Day
  - Event Recap Video: RDDC 2025 RISE Awards

- **Driving Inclusion, Advocacy, Policy, and Community Power in Rare Disease at the 2025 National Minority Quality Forum (NMQF) Leadership Summit**
    - RDDC partnered with NMQF for the Annual NMQF Leadership Summit on Health Disparities & Spring Health Braintrust, April 28–29, 2025 in Washington, DC. The summit is a premier event dedicated to advancing healthcare equity and plays a critical role in educating and mobilizing thousands of health leaders in the fight against disparities.
    - On April 29, RDDC led a 4-panel series focused on rare diseases (*links lead to individual panel recap videos*):
        - Diversity in Clinical Trials
        - Advancing Health Equity
        - Shaping Policy for Accessibility
        - Power of Community
    - **Event Program:** Driving Inclusion, Advocacy, Policy, and Community Power in Rare Disease at the 2025 NMQF Leadership Summit
    - **Full Event Recap Video:** RDDC Rare Disease Panels at the 2025 NMQF Leadership Summit
- **RDDC's First-Ever Capitol Hill Briefing**
    - On April 30, 2025, as part of its ongoing mission to advance equity in rare disease care and with the support of Senator Raphael Warnock of Georgia, RDDC called on stakeholders to take meaningful action. Through powerful testimony and groundbreaking survey data, RDDC highlighted persistent disparities faced by underrepresented communities. The coalition urged support for the HEARD Act of 2025 (H.R. 1750); encouraged partnerships; and advocated for amplifying patient voices, sharing findings, and staying engaged through RDDC's resources. This initiative was not just informative – it was a rallying cry to ensure all rare disease patients receive equitable diagnosis, treatment, and care.
    - **Event Program:** Addressing Health Disparities in Rare Disease: A Call to Action in Recognition of Minority Health Month
    - **Full Event Recap Video:** RDDC Capitol Hill Briefing April 2025
- **Successfully hosted the third round of the RDDC Fellowship Program** with one fellow hosted by American Muslim Health Professionals (AMHP). This fellowship program aims to inspire the next generation of the medical workforce, fostering a commitment to addressing rare disease disparities, particularly among people of color and in underserved and underrepresented communities. Additional details regarding the 2025 Spring Fellowship can be found on page 6 in the Patient-Provider-Caregiver Journey Workgroup section.

*Note: Throughout this report, you will find further elaboration on a handful of the items above, as well as other notable accomplishments.*

# WORKGROUP:
# Patient-Provider-Caregiver Journey

Rare disease patients and their caregivers are at the heart of RDDC's mission. To better serve these populations, we must gather data on the experiences of diverse patients with rare diseases and their caregivers. Caregivers, who often contribute selflessly and tirelessly to their loved ones' well-being, have historically been overlooked and inadequately supported. RDDC is committed to recognizing the unique needs and experiences of caregivers and patients, empowering them to advocate for their rights and well-being, and gathering insights to identify opportunities for systemic improvement.



## ACHIEVEMENTS

- In late August 2024, in partnership with National Organization of Rare Diseases (NORD), RDDC distributed the *Inequities in the Rare Disease Community: The Voices of Diverse Patients and Caregivers* Report. This report's primary objective was to close critical knowledge gaps and gain insights into these individuals' unique perspectives and challenges in accessing and affording health care. By concentrating on underrepresented patients, the intention was to gather essential information that would facilitate a more profound comprehension of existing gaps and barriers in diagnosis, care, and treatment access.
    - Two key recommendations from this report:
        - **Increase Access to Genetic Counseling:** Research suggests that approximately 72% of rare diseases are genetic in origin, however, fewer than half of our respondents have received genetic testing. Of those who did not receive testing, 85% were never offered it. There is an urgent need to ensure all individuals have access to affordable options for genetic counseling as part of their diagnostic process, particularly in communities historically underserved by the healthcare system. Therefore, clinicians across the healthcare system must be properly educated about these options and when and how to offer them.
        - **Expand Insurance Guidance and Coverage:** While most respondents have health insurance, a substantial proportion indicated their insurance did not allow them to access the providers, medication, procedures, and telehealth services they needed to manage their health. Access issues were reported more frequently by respondents from underrepresented communities. This highlights an opportunity for financial navigators to support rare disease patients – particularly in regions where more underrepresented people live – to educate them about existing options for co-pay assistance. We must advocate for more financial assistance programs where needs are not being met and for state governments to uphold the commitments of the Affordable Care and Patient Protection Act (ACA) to protect patients from inadequate health plans. Lastly, we can and must reform state and federal policies that create unnecessary bureaucratic barriers to care, particularly as it relates to care across state lines.
    - Press Release: Groundbreaking Study by RDDC and NORD: Unveiling Inequities in the Rare Disease Community Through the Voices of Diverse Patients and Caregivers
        - Visibility Report as of May 27, 2025:
            - Potential audience: 111 million
            - Exact matches picked up: 443
            - AP outlet distribution: 922 outlets
            - Release views: 1,682
            - Click-throughs: 244
    - Social Media Toolkit: *Inequities in the Rare Disease Community*

- In January of 2025, RDDC selected one new fellow. Launched in 2023, the RDDC Fellowship Program is designed to offer a transformative experience to current and future healthcare providers and researchers to bridge the knowledge gap in rare diseases. 2025 Spring Fellow Siwaar Abouhala embarked on a specialized fellowship with American Muslim Health Professionals (AMHP) focused on anti-discrimination research and implementation – more specifically, developing a curriculum for healthcare providers serving Muslim patients living with rare disease.
    - Siwaar Abouhala is a Clinical Research Coordinator on the Rare Genomes Project at the Broad Institute of MIT and Harvard. Siwaar's advocacy background is in Black and Brown birthing equity, having served as Founder of MARCH: Maternal Advocacy and Research for Community Health, the largest undergraduate-run maternal health organization in the United States, as well as a Research Assistant in the MOTHER Lab at Tufts Medicine and the NICU at Boston Children's Hospital. She graduated *summa cum laude* with highest thesis honors in May 2023 from Tufts University, where she triple majored in Arabic Language and Cultural Studies, Community Health, and Biology. She will be starting medical school in summer 2025 to become a physician-advocate.
    - Fellowship Blogs:
        - 3/14/2025: "Don't Forget the People: Re-Humanizing Rare Disease Research"
        - 5/1/2025: "The Social Determinants of Rare Disease: An Intersectional Lens"

- In early 2025, RDDC launched the Patient-Caregiver Advisory Board (PCAB) to create a safe, inclusive space for in-depth dialogue and to better understand the priorities of individuals navigating rare diseases. As an extension of the Patient-Provider-Caregiver Journey (PPCJ) Workgroup, PCAB is helping lead patient and caregiver-centered programming and outreach efforts. Its work is instrumental in shaping and elevating resources that directly support and reflect the lived experiences of diverse rare disease communities.

- RDDC is committed to educating and engaging with healthcare providers, as they are critical to bridging the gap in patient outcomes. In September of 2024, in partnership with the Bluff City Medical Society, we launched a 3-part webinar series titled *Improving Health Equity: Implications for Rare Disease*. This continuing medical education (CME) series explores the principles of health equity through the lens of rare diseases, with insights from international healthcare professionals and personal stories from rare disease patients and caregivers. The webinar recordings took place September through November 2024. The recordings are available for viewing at your convenience.
    - Participants will receive AMA PRA Category 1 credit(s) upon completing the program, and a certificate of completion will be provided to participants who meet all course requirements no later than December 31, 2025.
        - This FREE activity is approved for 1.5 hours of AMA PRA Category 1 credit(s) each for a total of 4.5 hours of CME for the entire series once completed. Feel free to share this information with colleagues to increase viewership.
    - Total Participants Completed as of May 2025:
        - Webinar 1 - 25 participants
        - Webinar 2 - 13 participants
        - Webinar 3 - 17 participants

- Press Release: The Bluff City Medical Society Foundation and Rare Disease Diversity Coalition Launch Three-Part Webinar Series on Improving Health Equity for Rare Diseases
  - Visibility report as of May 27, 2025:
    - Potential audience: 215 million
    - Exact matches picked up: 546
    - AP outlet distribution: 924 outlets
    - Release views: 2,033
    - Click-throughs: 16

## IN PROGRESS

- In the second half of 2025, RDDC will launch the "RISE for Rare" educational series. RISE for Rare is a storytelling-driven, educational initiative launched to elevate the lived experiences, cultural context, and unique challenges faced by historically marginalized and medically underrepresented communities impacted by rare diseases. Topics will include self-advocacy, medical gaslighting, and navigating relationships. The purpose of this series is to:
  - Humanize rare disease narratives by amplifying underrepresented voices.
  - Explore the intersections of race, gender, socioeconomic status, and healthcare access.
  - Educate patients, care partners, providers, and policymakers on barriers to culturally competent care and support services in addition to diagnosis and treatment.
  - Create opportunities for policy change, advocacy, and community engagement.
  - Offer tangible resources to support both everyday psychosocial needs and broader healthcare concerns.
- In 2025 Quarter 3, in partnership with Global Genes, RDDC will launch the next phase of the groundbreaking Rare Disease Curriculum designed to enhance education and awareness across healthcare, public health, and community sectors. During this phase, RDDC will focus on hosting several community pilots, obtaining accreditation, and utilizing the training content to draft more practical and digestible outreach materials. This curriculum was developed in 2024 with the goal of closing critical knowledge gaps, improving patient-provider communication, and equipping healthcare professionals and community health workers with the tools needed to better serve historically marginalized and medically underserved rare disease patients. A key differentiator of this program is its multi-audience accessibility – while designed for healthcare providers, community health workers, and medical teams, the curriculum is also structured in a way that makes it palatable for patients, caregivers, and community members, ensuring inclusive and equitable learning for all stakeholders in the rare disease ecosystem.
- In fall 2025, RDDC will announce the 2026 Rare Disease Fellow. The program will run for six months beginning in early 2026. This cycle features one new fellow who will be placed with a rare disease advocacy organization. Applications for the host site will be open in the fall of 2025.

# WORKGROUP:
# Government Regulation, Legislation and Policy

The RDDC's Government Regulation, Legislation, and Policy Workgroup are indispensable in confronting the many challenges underserved populations and their families face due to social determinants of health. A central objective of this workgroup is to advocate for regulatory measures within the national rare disease policy framework to meet these communities' distinctive needs. Our dedicated efforts encompass the meticulous crafting of policies and legislative initiatives to address the full spectrum of diagnosis, treatment, care, and support concerns.

## ACHIEVEMENTS

- **2025 RISE Awards: Congressional Recognition**
  - On February 27, 2025, RDDC was honored to present RISE Awards to Congressional leaders who have demonstrated exceptional dedication and leadership in advocating for rare diseases. This year's RISE Award honorees included Senator Amy Klobuchar of Minnesota, co-chair of the Rare Disease Caucus, who has been a legislative champion for rare disease patients through her co-sponsorship of the Accelerating Kids' Access to Care Act and introduction of the Speeding Therapy Access Today Act. Senator Bill Cassidy of Louisiana was also recognized for his longstanding advocacy on behalf of sickle cell warriors and for introducing the RARE Act, which seeks to extend drug exclusivity periods for rare pediatric diseases and therapeutics. These awards honor the unwavering commitment of Congressional leaders who have championed policies that enhance access to care, increase research funding, and strengthen patient support services – offering a beacon of hope to the rare disease community. By recognizing their contributions, RDDC seeks to inspire other lawmakers to follow their lead and advance legislative efforts that make a meaningful difference in the lives of those affected by rare diseases.
    - Event Recap Video: RDDC 2025 RISE Awards
      - Video Analytics as of June 2025:
        - Views: 41
        - Impressions: 532
    - Senator Amy Klobuchar Acceptance Video: U.S. Senator Amy Klobuchar Accepts the RDDC RISE Award
- **RDDC's First-Ever Capitol Hill Briefing**
  - On April 30, 2025, with the support of Senator Raphael Warnock of Georgia, RDDC reaffirmed its commitment to advancing equity in rare disease care by issuing a strong call to action. Drawing on compelling testimony and groundbreaking survey findings, RDDC shed light on the persistent disparities impacting historically marginalized communities. RDDC called for broad support of the HEARD Act of 2025 (H.R. 1750), emphasized the importance of strategic partnerships, and urged stakeholders to elevate patient voices, disseminate key insights, and remain engaged through RDDC's tools and initiatives. More than a briefing, this moment served as a catalyst – galvanizing efforts to ensure that all rare disease patients receive timely, equitable diagnosis, treatment, and care.
  - Event Program: Addressing Health Disparities in Rare Disease: A Call to Action in Recognition of Minority Health Month
  - Event Recap Video: RDDC Capitol Hill Briefing April 2025

- Sent a Congressional sign-on letter in recognition of Native American Heritage Month, calling for legislative action to support historically marginalized communities. The letter urged passage of H.R. 6533, the Urban Indian Health Parity Act, and H.R. 3305, the Black Maternal Health Momnibus Act – critical legislation that advances parity in healthcare delivery and supports research into the maternal health challenges facing American Indian and Alaska Native mothers. These efforts reflect RDDC's ongoing priority to center racial and ethnic equity within federal health policy and to advocate for policies that close longstanding access gaps.
- Met with bipartisan Congressional offices, including staff from the Senate Health, Education, Labor, and Pensions (HELP) Committee, the Make America Healthy Again Caucus, and the Rare Disease Caucus. These meetings helped amplify findings from RDDC's Barriers to Care Survey, a national effort to document the structural and systemic challenges rare disease patients and caregivers face when navigating the healthcare system. By proactively engaging policymakers with data-driven insights, RDDC aims to shape more informed legislation and promote patient-centered policy solutions at both the state and federal levels.
- Advocated for the extension of the FDA's Priority Review Voucher (PRV) Program, a critical incentive driving development of treatments for pediatric rare diseases. RDDC provided advocacy templates to workgroup members, equipping constituents to directly engage their Members of Congress in support of preserving this vital program. The PRV Program has played a pivotal role in accelerating the availability of lifesaving therapies for children with rare conditions – its continuation is essential to maintaining momentum in drug development and attracting private sector investment.
- Provided formal comments on the FDA's Industry Guidance for Diversity Action Plans, emphasizing the importance of a broader, intersectional view of diversity in clinical research. RDDC called for transparent demographic reporting, culturally responsive engagement materials, and long-term partnerships with trusted local organizations to build trust and boost enrollment from underrepresented populations. Our goal is to ensure that clinical trial participation reflects the full spectrum of patients affected by rare diseases and that innovations are tested in populations representative of those most in need.

## IN PROGRESS

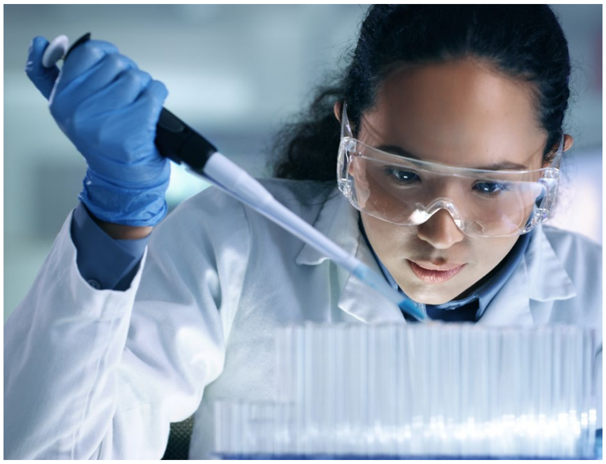
- RDDC continues to engage members of the Make America Healthy Again Caucus to ensure that national efforts to improve nutrition and prevent chronic disease meaningfully include the rare disease community. As public health legislation increasingly emphasizes preventive care and equity, RDDC is working to ensure that the diverse and complex needs of rare disease patients – many of whom face unique nutritional challenges and comorbidities – are not overlooked. This advocacy reinforces our belief that public health policy must reflect the full diversity of patient experiences, particularly for those in historically underserved communities.

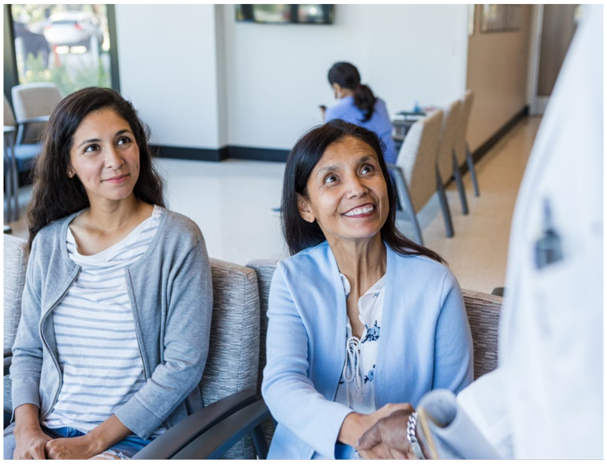
- Deepening bipartisan relationships with key Congressional leaders in healthcare remains a central focus of the Government Regulation, Legislation and Policy Workgroup. These relationships are not only strategic but foundational to advancing legislation that addresses the specific needs of rare disease patients. As RDDC builds institutional credibility and expands its coalition of allies on Capitol Hill, these connections will be critical in elevating rare disease priorities across a range of health policy agendas.

- Conducting ongoing analysis of Congressional and regulatory policy developments to identify legislative and administrative opportunities that align with RDDC's mission.
    - *The Inequities in Rare Disease Community* Report serves as a data-rich advocacy tool – enabling RDDC to spotlight systemic disparities in access to care, inform inclusive clinical trial design, and advance diversity-centered reforms in federal health programs. This analytical approach ensures that RDDC's policy engagement is both evidence-based and rooted in the lived experiences of the communities we serve.

# WORKGROUP:
# Diversity in Research and Clinical Trials

Clinical trials of potential treatments for rare diseases are vital to identifying effective patient interventions. Given considerable health disparities in rare diseases, it is vital that including diverse populations is prioritized in the recruitment and selection of clinical trial participants.[2] This commitment stems from the understanding that clinical trials must accurately reflect the heterogeneous nature of rare disease patients, ensuring the safety and effectiveness of treatments once approved. RDDC's focus on clinical trials and the equitable inclusion of historically marginalized populations sets us apart and positions us as a beacon of change and progress.





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[2] Informed DNA "The importance of achieving diversity in rare disease clinical trials". April 20, 2021. Retrieved from https://informeddna.com/blog-achieving-diversity-in-rare-disease-clinical-trials/

## ACHIEVEMENTS

- In partnership with the Indo US Rare Disease Organization, RDDC is proud to announce the publication of two groundbreaking manuscripts, currently in the final stages of peer review:
    - *Current State and Demographic Trends of Medically Underserved Populations in Rare Disease Research in the United States*
    - *A Framework for Inclusive and Accessible Clinical Research in Rare Diseases*

    These manuscripts explore the intersection of Diversity, Equity, Inclusion, and Accessibility (DEIA) principles with the complex realities faced by medically underrepresented and marginalized communities affected by rare diseases. Recognizing the scope and impact of this work, RDDC provided strategic support to expand our partner's research capacity, helping to secure additional talent to advance this critical initiative. Together, these studies offer data-driven insights; identify persistent inequities; and provide actionable recommendations to address systemic barriers across race, ethnicity, socioeconomic status, and other social determinants of health. This collaboration underscores our shared commitment to transforming clinical research landscapes and to ensuring that no rare disease community is left behind.

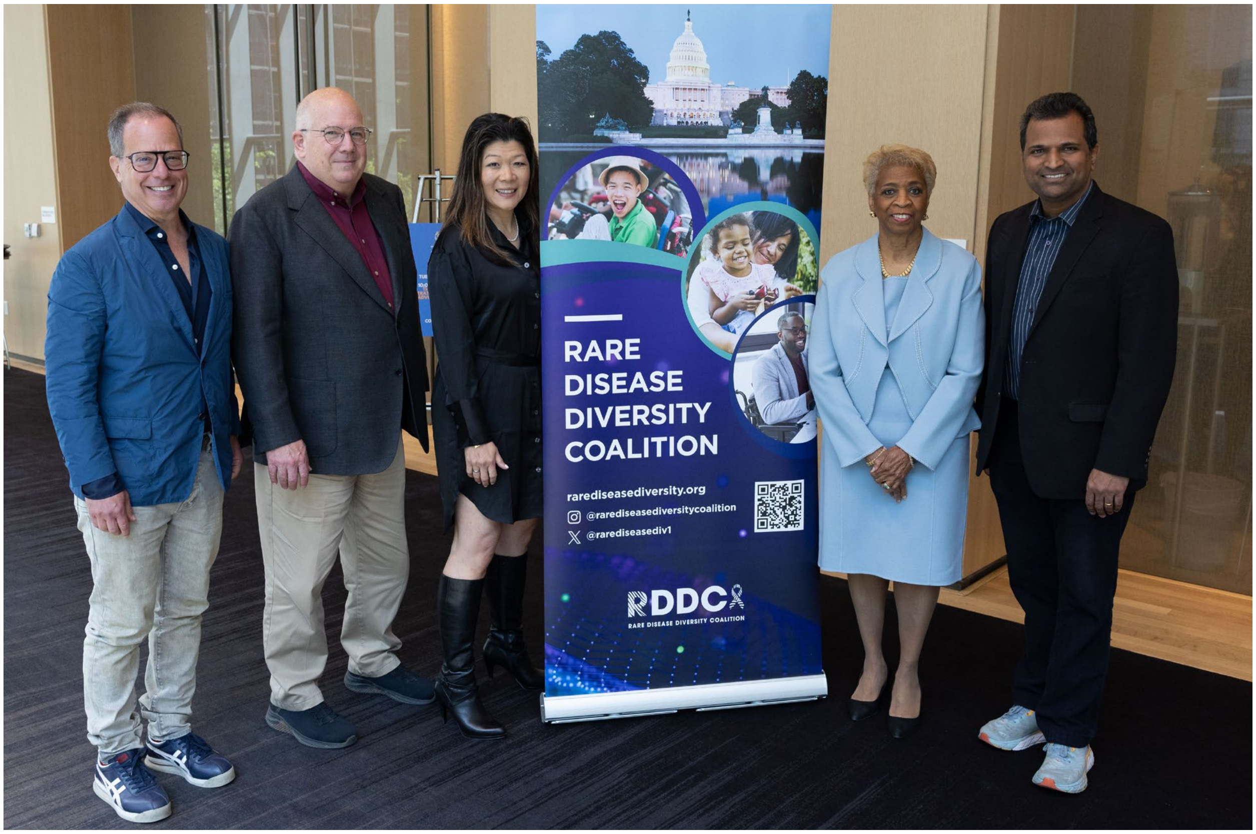
- Through a strategic partnership between RDDC and The Alliance to Cure Cavernous Malformations, the initiative "Equipping Community Health Workers to Increase Clinical Trial Understanding among Hispanic New Mexicans" aims to advance health equity and inclusivity. By enhancing the capacity of community health workers (CHW), the project is improving awareness and understanding of rare disease clinical trial processes within Hispanic communities in New Mexico, fostering broader and more equitable participation in clinical research and expanding access to potential treatments. This program will use a 3-step approach:
    - First, a comprehensive New Mexico CHW training program to target CHW awareness of clinical trials and rare disease clinical trials, thereby reducing healthcare disparities. Emphasizing inclusivity, it empowers CHWs to disseminate accurate information, fostering informed decision-making.
    - Second, a talk radio initiative on local radio amplifies awareness on clinical trials, research participation, and cavernous malformations (CCM) in rural areas, fostering trust and encouraging participation.
    - The third approach introduces a transformative Progressive Web App (PWA) tailored for CHWs, providing instant access to resources and education on clinical trials, including information on CCM, other rare disease resources, and clinical trials directories.

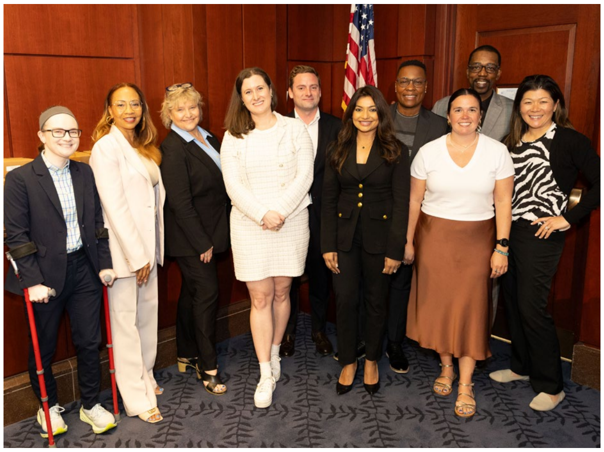
## IN PROGRESS

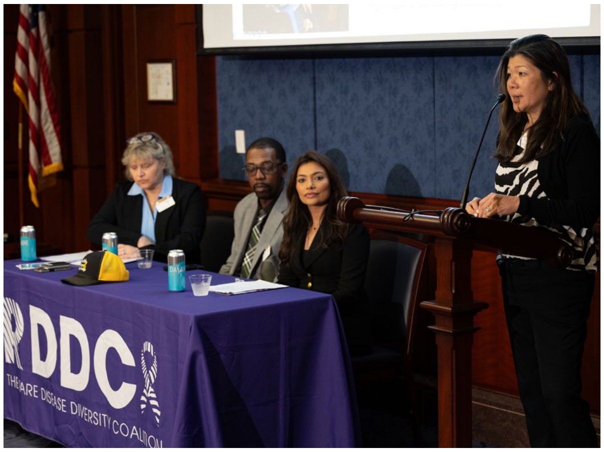
- A comprehensive review of the Rare Disease Clinical Resource Network (RDCRN) diversity demographics data collected from clinical trial sites associated with the National Institutes of Health (NIH) over the past 20 years is approaching publication in fall 2025. This review provides critical insights into the representation and participation of diverse populations in rare disease clinical trials – highlighting trends, gaps, and a baseline of information for future reference. This review is a critical step toward understanding and addressing the disparities in clinical research participation.
- The Breaking Barriers to Genetic Testing Expansion Initiative aims to increase equitable access to genetic testing among BIPOC, LGBTQIAP+, rural, and underinsured communities disproportionately impacted by rare diseases. By addressing the cultural, systemic, and economic barriers to testing, this initiative supports earlier diagnoses, improves clinical decision-making, and fosters greater trust in precision medicine.

# THE POWER OF PRESENCE:
# Advancing Rare Disease Awareness Through Conferences

Presenting at conferences plays a vital role in our mission to accelerate progress in rare disease research and care. These opportunities allow us to share groundbreaking insights, elevate the voices of patients and caregivers, and foster collaboration with global experts. By engaging with the broader medical and scientific community, we raise awareness and spark innovation and partnerships that drive meaningful change for those affected by rare diseases.







- **Critical Path Institute Rare and Orphan Disease Programs Webinar Series**

  On October 29, 2024 speakers Jenifer Waldrop - Executive Director, Rare Disease Diversity Coalition (RDDC) and special guest Nate Milam - Histio Ambassador and RDLA Committee Member, discussed the RDDC national survey, *Inequities in the Rare Disease Community: The Voices of Diverse Patients and Caregivers,* aimed at illuminating the barriers faced by underrepresented patients with rare diseases.

- **21st Annual World Symposium**

  During the 21st Annual World Symposium, February 3–7, 2025, there was a 1-hour CE-session titled, "Patient Voice 2025: Inequities in Access to Diagnosis, Care and Clinical Trials in Lysosomal Diseases". This session featured RDDC Executive Director Jenifer Waldrop and addressed population inclusion and access to care and clinical trials regardless of diversity and socioeconomics, with a focus on improving patient care globally. The speakers strived to recognize individual perspectives and struggles for families despite backgrounds and socioeconomic and racial barriers, and to identify realistic ways to improve opportunities for all patients.

- **Autoimmune Network Monthly Webinar - Guest Speaker: Jenifer Waldrop from the Rare Disease Diversity Coalition**

  The Autoimmune Registry hosts a monthly webinar to bring together patient advocacy groups related to autoimmune diseases. During the February 20th webinar, Jenifer Waldrop joined Reggie Barnes, Director of Patient Partnerships at the Autoimmune Registry, to discuss the *Inequities in the Rare Disease Community* report.

- **APAICS Health Summit**

  March 25–26, 2025, the 2025 APAICS Health Summit aired virtually. The summit brought together frontline workers, community and corporate leaders, subject matter experts, and elected officials for bipartisan policy discussions on issues that affect our communities and the nation at large. The Summit explored topics such as representation in healthcare and research, and healthcare accessibility for the AA & NH/PI community.

- **World Orphan Drug Congress**

  During the World Orphan Drug Congress, April 22–24, 2025, top pharmaceutical and biotech companies, government and regulatory authorities, patient advocacy groups, payers, investors and solution providers convened in Boston. This conference provided a forum to discuss and advance orphan drug development and enhance access to life-saving therapies for individuals living with a rare disease.

- **Myasthenia Gravis Awareness Day**

  On June 7, 2025, RDDC had the opportunity to attend the MG Awareness Day & Health Summit, hosted by the MG Holistic Society at the historic Academy of Medicine in Midtown Atlanta. The event was a vibrant celebration of wellness, education, and community connection for individuals living with myasthenia gravis and their caregivers.

  - The summit featured dynamic speakers sharing holistic health strategies, local vendors, and resources from community organizations. As participants, we were proud to engage with a diverse and empowered community and to support the MG Holistic Society's efforts to promote culturally responsive, patient-centered care. Additionally, RDDC also used this opportunity to connect directly with MG audiences to collect valuable data through our community survey sponsored by Amgen. The insights we gathered will help inform ongoing advocacy, education, and research efforts focused on addressing disparities in rare disease care.

- **Healthcare Improvement Trailblazers (HIT) Summit**

  The second edition of the Healthcare Improvement Trailblazers (HIT) Summit took place in Washington, DC June 25–27, 2025. Participants included healthcare providers; payers: the biopharmaceutical, MedTech and diagnostics industry, patients, and patient organizations. They all came together to discuss quality and care improvement as well as continued learning and education. RDDC Executive Director Jenifer Waldrop participated in this event as a panel moderator and presenter.

## Featured Publications: Inspiring Progress in Rare Disease

These publications, in addition to the press releases shared earlier throughout the report, serve as a testament to the quality and relevance of the work, demonstrating thought leadership, expertise, and influence. We present a selection of our most notable publications, showcasing our commitment to excellence and innovation. They have been carefully chosen for their impact, significance, and relevance to current trends and challenges in our field. By sharing these works, we aim to demonstrate our ongoing dedication to advancing knowledge, fostering collaboration, and making a meaningful impact in our community and beyond.

- Strickland Boosts Rare Disease Research for Underserved and Minority Patients. *Office of Congresswoman Marilyn Strickland*. Published February 28, 2025. https://strickland.house.gov/2025/02/28/strickland-boosts-rare-disease-research-for-underserved-and-minority-patients/
- Health organizations collaborate to address idiopathic pulmonary fibrosis on Rare Disease Day 2025. *News-Medical.net*. Published February 28, 2025. https://www.news-medical.net/news/20250228/Health-organizations-collaborate-to-address-idiopathic-pulmonary-fibrosis-on-Rare-Disease-Day-2025.aspx
- Rare Diseases at Crossroads: Building on 42 years of progress powered by the Orphan Drug Act. *EIN Presswire*. Published March 11, 2025. https://www.einpresswire.com/article/789245614/rare-diseases-at-crossroads-building-on-42-years-of-progress-powered-by-the-orphan-drug-act
- Jenifer Ngo Waldrop of Rare Disease Diversity Coalition (RDDC). *RARE Revolution Magazine*. Published June 5, 2025. https://rarerevolutionmagazine.com/jenifer-ngo-waldrop-of-rare-disease-diversity-coalition-rddc/.

# CONCLUSION

RDDC remains deeply committed to creating lasting, meaningful change in the lives of rare disease patients and their families – especially those from historically underrepresented communities. Our achievements in 2024–2025 reflect the collective dedication of our team, partners, and supporters who share in this mission.

We are grateful for your continued partnership. As we look toward 2026 and beyond, we are energized to keep driving progress in rare disease awareness, education, and timely diagnosis – together with committed allies like you.

**If you are interested in our mission and want to get involved, please check out our website at [rarediseasediversity.org](https://rarediseasediversity.org), or email our team at [rddc@bwhi.org](mailto:rddc@bwhi.org).**

RDDC

RARE DISEASE DIVERSITY COALITION

www.rarediseasediversity.org