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Annual Policy Report

2025

Navigating a New Era in Federal Healthcare

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The inauguration of President Donald Trump and the swearing in of the Republican-led 119th Congress marked the beginning of a sweeping transition in federal healthcare policy. With new leadership installed across the major health agencies, the Administration immediately signaled a dramatic shift toward government-wide restructuring, reducing regulatory burdens, and reshaping core federal health priorities.

At the same time, the 119th Congress launched an ambitious agenda centered on realigning federal spending and advancing legislation to support the Administration's priorities, most notably the One Big Beautiful Bill Act (OBBB – H.R.1 or the "reconciliation package"), which made significant changes to the federal tax code as well as the Medicaid program. Together, these changes reflected a coordinated effort to reshape the federal government's role in healthcare by reducing federal funding for many mandatory and discretionary programs, eliminating equity initiatives and operationalizing market-driven models focused on transparency requirements and voluntary compliance. These actions collectively signal a significant shift in federal healthcare policy priorities.

In order to give Rare Disease Diversity Coalition (RDDC) members and rare disease stakeholders a clear understanding of federal and congressional developments with the greatest implications for rare disease research, early diagnosis, therapeutic innovation, and long-term access to care heading into 2026, RDDC has prepared this Annual Report. The Annual Report does not catalog every federal and congressional action taken over the past year. Instead, it highlights the most consequential policies, programs, and legislative decisions that reshaped the landscape in which rare disease patients, providers, researchers, and advocates must operate. The analysis focuses on major administrative actions, structural reforms across federal health agencies, key regulatory shifts, significant congressional activity, and the broader policy environment shaped by the 2025 reconciliation package and the resolution of the longest government shutdown in U.S. history.

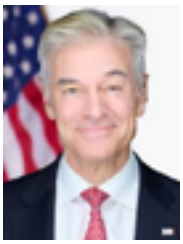
Administrative and Executive Branch Developments

New Healthcare Leadership and Direction of Federal Health Agencies

President Trump entered office in January 2025 with a goal of reshaping federal healthcare policy around lower costs, increased transparency, deregulation and "America First" industrial and trade priorities. These commitments were reflected immediately in the President's early appointments across the federal health agencies. Each appointee brought a distinct perspective on regulation, research, and public health delivery, collectively reinforcing the President's desire for dramatic shifts in focus across the U.S. Department of Health and Human Services (HHS).

Robert F. Kennedy Jr.
Secretary of HHS

Kennedy, long known for his skepticism toward federal public health institutions and vaccines, as well as his interest in the environmental drivers of disease, took the helm at HHS and focused on large-scale restructuring, workforce reductions, and consolidation of federal health programs. As Secretary, Kennedy has led the Administration's work to Make America Healthy Again (MAHA) through his leadership of the MAHA Coalition, among other items.



Dr. Mehmet Oz
Administrator of the Centers for Medicare & Medicaid Services (CMS)

A cardiothoracic surgeon and former television medical host, Dr. Oz's leadership emphasized consumer empowerment, competition, and price transparency. His approach positions CMS to pursue payment reforms and demonstration models that reward prevention, expand patient choice, and reduce regulatory burden.

Dr. Martin Makary
Commissioner of the U.S. Food and Drug Administration (FDA)

Dr. Makary, a surgeon and public health scholar recognized for his advocacy around medical transparency and improving efficiency within medical practice, has moved quickly to accelerate regulatory review processes for drugs including those for rare diseases. Early initiatives include expanding the use of artificial intelligence (AI) in FDA reviews, modernizing guidance for cell and gene therapies and establishing the Commissioner's National Priority Voucher (CNPV) Program.

Dr. Jay Bhattacharya
Director of the National Institutes of Health (NIH)

A physician-economist and public health researcher known for his critique of the federal government's response to the COVID-19 pandemic, Dr. Bhattacharya entered NIH committed to rethinking the agency's structure and realigning research priorities. His tenure has coincided with proposals to consolidate multiple NIH institutes and reduce NIH funding.

Executive Actions and Major Shifts in Health Policy

In its first year, the Administration relied extensively on executive orders (EOs) to rapidly shift federal healthcare priorities. While these directives varied in scope, several exerted broad influence over health insurance markets, drug prices, and sought to realign the frameworks that support rare disease care. Collectively, these actions reflect a governing approach focused on cost containment, transparency, regulatory streamlining, and redefining federal efforts on equity initiatives.

For the rare disease community, this landscape creates both opportunities and vulnerabilities: transparency and efficiency measures may ease care navigation, but drug-pricing reforms, reductions in equity-focused policy, and shifts in federal public health infrastructure may undermine long-term research stability and sustained access to specialized therapies. Below are notable EOs impacting healthcare.

01

EO 14151

Ending Radical Wasteful Government DEI Programs and Preferencing

January 20, 2025

This EO eliminates DEI programs and related requirements across federal agencies, including diversity action plans, reporting obligations and equity-focused program design. While not healthcare-specific, it significantly narrows the federal emphasis on identifying and addressing disparities. Because rare diseases disproportionately affect individuals who already face delayed diagnosis, particularly minority, rural, and underserved populations, the rollback of DEI and indefinite pause on advancing diversity action plan requirements risk reducing attention to structural barriers in early detection, clinical trial inclusion, care coordination, and access to specialized services.

02

EO 14168

Defending Women from Gender Ideology Extremism and Restoring Biological Truth to the Federal Government

January 20, 2025

This EO directs federal agencies and federal employees to interpret "sex" solely as an immutable binary biological classification determined at conception. The order also requires all federal agencies to enforce sex-based rights, protections, and accommodations using this definition of "sex." The order also instructs agencies to "evaluate grant conditions and grantee preferences" to ensure federal funds are not used to "promote gender ideology." The order has implications for LGBTQ individuals, particularly transgender and intersex patients, by limiting access to gender-affirming care and narrowing the circumstances under which discrimination claims can be brought.

03

EO 14212

Establishing the President's Make America Healthy Again Commission

February 13, 2025

This EO establishes the MAHA Commission to advise and assist the president in addressing the prevalence of chronic disease among children. The MAHA Commission's May 22 inaugural report identified four main drivers of chronic disease in the U.S.: poor nutrition, chemical exposure, lack of physical activity, and chronic stress, as well as overmedicalization, as root causes of chronic childhood diseases such as obesity and diabetes.

Most notably, the report discusses an agency restructuring at HHS to create the Administration for a Healthy America (AHA), which will combine multiple agencies—the Office of the Assistant Secretary for Health (OASH), Health Resources and Services Administration (HRSA), Substance Abuse and Mental Health Services Administration (SAMHSA), Agency for Toxic Substances and Disease Registry (ATSDR), and National Institute for Occupational Safety and Health (NIOSH)—into a singular, centralized agency focused on primary care, maternal and child health, environmental health, HIV/AIDS, and workforce development.

04

EO 14297

Delivering Most-Favored National Prescription Drug Pricing to American Patients

May 12, 2025

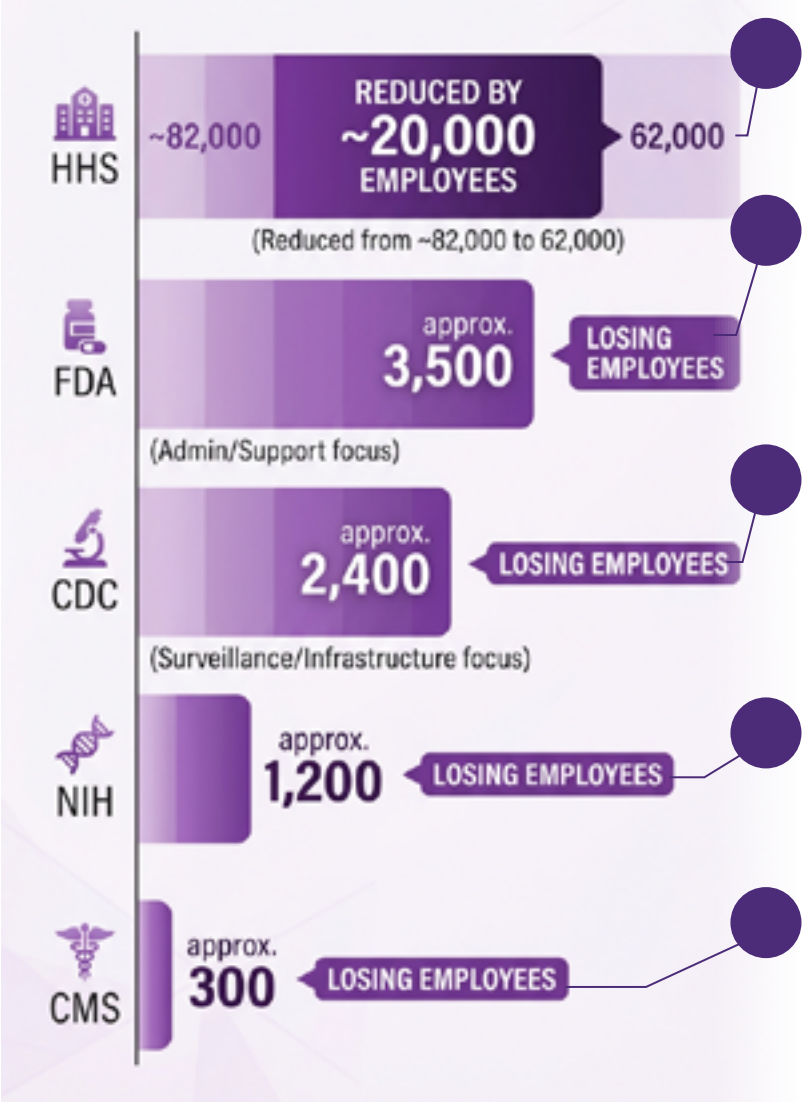
This EO instructs HHS, the Department of Commerce and the U.S. Trade Representative (USTR) to pursue Most-Favored-Nation (MFN) drug pricing strategies to reduce the cost of prescription drugs in the U.S. to levels paid for in other countries. For rare diseases, MFN pricing may introduce substantial risk, as manufacturers may limit product launches, delay U.S. availability, or reduce investment in rare disease pipelines if price ceilings undermine cost recovery. The Administration has since pursued drug pricing agreements with individual pharmaceutical manufacturers.

Federal Reductions in Force Across Health Agencies

The Administration positioned the reduction of federal healthcare agencies as a central component of its broader goal to "right-size" government and limit the federal role in healthcare delivery, research, and oversight. By consolidating agencies, eliminating positions, and narrowing administrative functions, the Administration aims to create a smaller, more efficient federal footprint. While efforts to reorganize federal healthcare agencies may require an act of Congress to fully operationalize, reductions in force (RIFs) posed meaningful risks for rare disease. Rare disease diagnosis, research, therapy development, and access often depend on the very federal programs undergoing reductions in workforce. Large-scale RIFs can weaken program continuity, slow regulatory timelines, reduce research support, and destabilize the infrastructure that rare disease patients and providers rely on more heavily than other populations.

> "Large-scale RIFs can weaken program continuity, slow regulatory timelines, and destabilize the infrastructure rare disease patients rely on."

Agency-by-Agency Summary of Announced Reductions in Force



U.S. Department of Health and Human Services (HHS):
Announced a sweeping workforce reduction from roughly 82,000 to 62,000 full-time employees through layoffs, retirements and buyouts.

U.S. Food and Drug Administration (FDA):
Expected to lose approximately 3,500 employees. While HHS and FDA leadership have emphasized that core drug and device review staff will not be included in RIFs, major reductions will occur across administrative and support offices.

Centers for Disease Control and Prevention (CDC):
CDC and related preparedness offices are expected to see significant workforce reductions of approximately 2,400 employees, affecting surveillance, data systems, public-health infrastructure, and community-based programs.

National Institutes of Health (NIH):
Slated for a reduction of roughly 1,200 employees as part of broader structural consolidation and a shift toward fewer institutes with broader mandates.

Centers for Medicare & Medicaid Services (CMS):
Estimated reduction of around 300 employees. Though the agency states program operations will remain fully functional, reduced staffing will impact administrative functions and oversight capacity.


RDDC

Key Agency Actions

Food and Drug Administration (FDA)

- **CNPV Pilot Program:**
  On June 17, 2025, the FDA announced the establishment of the CNPV Pilot Program. Under the program, drug and biological product developers may receive a voucher that can be used to accelerate FDA product application or efficacy supplement review times from 10-12 months to one to two months. Vouchers under the CNVP program will be awarded to companies that align with U.S. national priorities, including: addressing a health crisis in the U.S., delivering more innovative cures for the American people, addressing unmet public health needs, and increasing domestic drug manufacturing as a national security issue.

  The FDA announced more than 15 awardees to date. Awardees included drugs that treat rare diseases, including CASGEVY® for sickle cell disease and Zongertinib for HER2 lung cancer.

- **Draft Guidance on Gene and Cell Therapies:**
  The FDA published three draft guidance documents on September 25, 2025, related to the development of cell and gene therapies (CGTs) to treat rare diseases, post-approval studies of CGTs, and expediting the time it takes for regenerative medicines to reach the market.

  Draft guidance, "Innovative Designs for Clinical Trials of Cellular and Gene Therapy Products in Small Populations," provides recommendations to clinical trial sponsors who intend to conduct clinical trials involving cell and gene therapy products intended for use in a rare disease or condition that impacts a small population. Recommendations include diverse clinical trial designs, as well as potential consideration of the entire treatment landscape and a patient's overall symptoms and whether symptoms are overall representative of a particular disease when deciding whether to enroll patients.

  Draft guidance, "Post approval Methods to Capture Safety and Efficacy Data for Cell and Gene Therapy Products," discusses methods and approaches to capture post-approval safety and efficacy data for CGT products. The guidance seeks to develop new methods for capturing adverse event data and encourages sponsors to review real-world data and evidence through sources such as electronic health records (EHRs), claims data, and other sources.

  Draft guidance, "Expedited Programs for Regenerative Medicine Therapies for Serious Conditions," provides guidance to clinical trial sponsors developing regenerative medicine therapies for serious or life-threatening conditions. The 21st Century Cures Act allowed regenerative medicine advanced therapies (RMATs) to use FDA's accelerated approval pathway. The guidance seeks to outline how RMATs may qualify for the pathway and potentially the FDA's Platform Technology Designation Program.

- **Expanded Access Final Guidance:**
  In October 2025, the FDA released a revised guidance titled, "Expanded Access to Investigational Drugs for Treatment Use — Questions and Answers." The document provides detailed instructions on how and when patients may access investigational products to treat serious or life-threatening conditions when no satisfactory alternatives exist. The document also details intellectual property management and data protection strategies. The FDA reiterates that expanded access (also known as "compassionate use" and/or "preapproval access") is intended primarily for treatment rather than data collection and remains subject to the same safety and oversight requirements as applicable to investigational new drugs. The guidance confirms that three expanded access pathways are available: individual patient use (including emergency use), intermediate-size population programs, and treatment investigation new drug applications (INDs) or protocols for larger groups.

Centers for Medicare & Medicaid Services (CMS)

- **Calendar Year 2026 MA and Part D Final Rule:**
  On April 4, 2025, CMS issued a final rule outlining changes to the Medicare Advantage (MA) and Medicare Prescription Drug (Part D) programs for plan year 2026. The proposed rule, released in November 2024 under the previous administration, included a broad range of provisions. However, CMS is finalizing only a select number of policies. The rule finalized proposed clarifications requiring MA and Part D plans to honor medical necessity decisions rendered as part of a prior authorization process, closed loopholes in MA appeals processes by explicitly defining organizational determinations eligible for appeal, and codified requirements designed to improve enrollee experience interacting with dual eligible special needs plans. Additionally, the rule finalized proposals regarding vaccine and insulin cost sharing for Part D plans and requires all Part D plans to require network pharmacies to be enrolled in the Medicare Drug Price Negotiation Program's Medicare Transaction Facilitator Data Module (MTF DM).

Health Resources and Services Administration (HRSA)

- **340B Rebate Model Pilot Program:**
  The 340B Drug Pricing Program, designed to help safety-net providers access discounted outpatient drugs, is undergoing a significant shift with the launch of HRSA's voluntary 340B Rebate Model Pilot Program. Beginning January 1, 2026, this pilot is intended to allow drug manufacturers to offer post-sale rebates—instead of traditional up-front discounts—for select drugs, notably those subject to Medicare Drug Price Negotiation. The Program is currently on hold pending litigation.

Congressional Updates

The legislative landscape in 2025 was marked by fiscal tension, a record-setting government shutdown and the passage of a sweeping reconciliation package that made significant changes to the Medicaid program. Much of the year revolved around debate over the future of the Affordable Care Act's enhanced Advance Premium Tax Credits (APTCs), which have become central to maintaining affordable marketplace coverage. When negotiations stalled, the government entered a 43-day shutdown, the longest in U.S. history. The impasse ended only after Senate Majority Leader John Thune pledged to hold a Senate vote on APTCs before year's end, securing enough Democratic support to reopen the government. No similar commitment was made in the House, leaving the policy's long-term fate uncertain.

Alongside the passing of a major reconciliation package that extended tax cuts from the 2017 Tax Cuts and Jobs Act (TCJA) and made substantial changes to Medicaid, Children's Health Insurance Program (CHIP), Medicare, and the Affordable Care Act (ACA), Congress pursued narrower initiatives on rural health, pediatric access, telehealth, payment reforms, and pharmacy benefit manager oversight. Despite broader partisan divides, several targeted health bills advanced with bipartisan support, reflecting shared interest in addressing workforce shortages, care access, and delivery challenges.

The shutdown highlighted deep divisions over coverage affordability, while reconciliation set in motion far-reaching reforms that federal and state agencies will implement in coming years that will have lasting consequences for the rare disease community and the broader healthcare system.

H.R. 1, The Reconciliation Bill

Congress approved the 2025 reconciliation package in July 2025. The sweeping package extends key tax cuts from the 2017 tax law and makes substantial changes across major federal healthcare programs, including Medicaid, CHIP, Medicare, and the ACA. The Congressional Budget Office (CBO) estimates the law will cut federal Medicaid spending by $911 billion over 10 years and will increase the number of people who are uninsured by 10 million in 2034.

For the rare disease community, these reforms carry meaningful implications. Reductions in federal Medicaid funding and changes to state flexibility in operating their Medicaid programs may influence how medically complex patients receive care, many of whom rely on Medicare or Medicaid – or both. As states receive less funding from the federal government, it is likely states will have to make difficult decisions regarding formulary coverage, benefits, and other changes to offset potential funding losses. In addition, adjustments to Medicare payment and drug policies under the Medicare Drug Price Negotiation Program may address affordability and availability of high-cost specialty therapies while shifts in ACA subsidy structures and marketplace rules could alter the stability of coverage that families rely on to obtain timely diagnostics and care. Taken together, these reforms reshape the underlying structure and financing of core health programs and set the stage for extensive federal and state implementation activity. Below are some of the key changes.

$911

BILLION

Reduction in federal Medicaid spening over 10 years

10

MILLION

Projected increase in uninsured insured individuals by 2034.

Medicaid/CHIP

- **"Community Engagement" Requirements:**
  Able-bodied adults must affirm monthly that they spend no less than 80 hours per month working, participating in a work program, completing community service, participating in an educational program, or participating in a combination of those activities. Exceptions are made for certain individuals including those under 19 years of age and individuals experiencing certain short-term hardship events. The CMS recently issued guidance to states regarding implementation of this policy.

- **Limits Certain Noncitizen Access to Federal Health Services:**
  Prevents certain non-citizens from enrolling in or receiving benefits under Medicaid/CHIP.

- **Eligibility Redeterminations:**
  To be conducted every six months, starting on or after December 31, 2026.

- **Limits Payments to "Prohibited Entities":**
  No federal funding may be used to make payment to prohibited entities, including 501(C)(3) nonprofit organizations, essential community providers primarily engaged in providing family planning services, reproductive health, or related services, or entities that provide abortions except under certain circumstances.

- **Increased Cost Sharing:**
  Beginning October 1, 2028, requires states to impose cost-sharing requirements or levy similar charges totaling no more than $35 for certain services, care, or items furnished to Medicaid enrollees, excluding primary care, mental health, substance use disorder services, services provided by federally qualified health centers (FQHCs), certified community behavioral health centers (CCBHCs), and rural health clinics. Cost sharing may not exceed 5 percent of a family's income.

Medicare

- **Limits Certain Noncitizen Access to Federal Health Services:**
  Prevents certain noncitizens from receiving premium tax credits to reduce the cost under Medicare.

- **Exemption of Orphan Drugs from Medicare Drug Price Negotiation:**
  The Inflation Reduction Act (IRA) exempted orphan drugs – medicines designed to treat rare diseases – from being subject to the Medicare Drug Price Negotiation Program if the drug was intended to treat only a single indication or condition. H.R.1 expands the exemption to include orphan drugs approved for more than one rare disease starting in 2028. The language also extends the time that orphan drugs may be exempt from Medicare Negotiation if a nonrare disease indication is added to a drug.

ACA

- **Limits Certain Noncitizen Access to Federal Health Services:**
  Limits the availability of premium tax credits (PTCs) for plans through the ACA marketplaces to certain noncitizens, disallows the availability of PTCs during the time certain noncitizens are not eligible for Medicaid, and requires monthly verification of eligibility for receipt of PTCs.

- **Enhanced Advanced Premium Tax Credits.**
  The bill does not extend enhanced advanced premium tax credits (APTCs) that expired at the end of 2025.

Other Provisions

- **Rural Health Transformation (RHT) Program:**
  H.R.1 established the RHT Program which will provide $50 billion to states over five years, with $10 billion of funding available each fiscal year between FY26 and FY30. $25 billion will be distributed among states whose applications are approved while the remaining $25 billion will be allocated by CMS based on a variety of factors, including rural population, the proportion of rural health facilities in the State, the situation of certain hospitals in the State and other factors identified by CMS. The application window for states closed on November 5, 2025, and funding allocations were announced at the end of 2025. States must use RHT Program funds to drive innovative solutions to transform rural health, through workforce initiatives, training, improving preventative functions, and increasing access to substance abuse and mental health treatments.

- **Expanded Access to Health Savings Accounts:**
  H.R.1 expands access to HSAs for patients with high-deductible health plans (HDHPs) and patients with bronze and catastrophic-level plans through the ACA marketplaces. HSAs allow individuals to put pre-tax dollars into an HSA for use in paying for qualified medical expenses. An unused HSA may be rolled over year over year. Under H.R.1, patients with HSAs under newly eligible plans may also now use HSAs to pay for direct primary care, which is when patients pay a set monthly or annual fee to their primary care physician instead of having care reimbursed through their health insurance plan. In addition, H.R.1 includes a provision allowing telehealth services to be covered on a pre-deductible basis for individuals enrolled in HDHPs.

Legislative Updates

Even with much of Congress's attention devoted to the shutdown and the reconciliation package, lawmakers continued to advance a range of more focused health policy initiatives. Measures related to rural health, pediatric care, telehealth expansion, payment modernization, and pharmacy benefit manager reform all moved forward, demonstrating that certain healthcare priorities still garner bipartisan interest. These targeted efforts underscore ongoing concern about workforce capacity, access to specialty services, and the stability of care delivery, even amid a highly polarized legislative environment.

Became Law

- **SUPPORT for Patients and Communities Reauthorization Act of 2025 (Pub. Law No. 119-44, H.R.2483):**
  The law renews funding for drug prevention and treatment programs as well as mental health initiatives enacted in 2018 under the bipartisan SUPPORT for Patients and Communities Act (Pub. Law 115-271).

Passed One Chamber

- **The Mikaela Naylon Give Kids a Chance Act (H.R. 1262):**
  Passed by the U.S. House of Representatives, reauthorizes the FDA's Rare Pediatric Disease Priority Review Voucher (PRV) Program through 2029, strengthens FDA authority to enforce pediatric study requirements, and clarifies the scope of orphan drug exclusivity. Together, these provisions aim to support pediatric and rare disease drug development while providing regulatory predictability for sponsors and patients.

- **Chronic Disease Flexibility Act (H.R.919):**
  Provides statutory authority for guidance from the Internal Revenue Service (IRS) that expands the types of preventive care that may be offered under a high-deductible health plan (HDHP) without requiring a deductible or with a deductible below the minimum threshold.

- **PROTECT Rare Act (S.3551):**
  Introduced in the Senate to expand coverage of off-label drug use for rare diseases by allowing Medicare, Medicaid, and private insurers to rely on clinical evidence and expert guidance when making coverage decisions. The bill aims to amend titles XVIII and XIX of the Social Security Act and title XXVII of the Public Health Service Act to provide for coverage of certain drugs used in the treatment or management of a rare disease or condition, and for other purposes.

Introduced

- **Health Equity and Rare Disease Act (HEARD) Act (H.R.1750):**
  Establishes grants to public and private nonprofits, health professional schools, and tribal and urban health centers for data collection, public outreach, and treatment of rare diseases and would establish loan repayment and forgiveness for certain medical professionals treating rare diseases.

- **Cameron's Law (H.R.1414):**
  Increases the Orphan Drug Tax Credit to 50 percent (from 25 percent) of qualified clinical testing expenses paid or incurred in the development of drugs to treat certain rare diseases or conditions.

- **New Era of Preventing End-Stage Kidney Disease Act (H.R.1518):**
  Establishes regional centers of excellence, postgraduate fellowships, and training for health professionals relating to the diagnosis and treatment of rare kidney disease. It also requires HHS to conduct various studies on rare kidney disease.

- **National Plan for Epilepsy Act (H.R.1198, S.494):**
  Directs the Secretary of HHS to establish a national plan to coordinate research on epilepsy and an Advisory Council on Epilepsy Research, Care and Services.

- **Women and Lung Cancer Research and Preventive Services Act (H.R.2319):**
  Directs the Secretary of HHS to conduct a review to evaluate the status of research on lung cancer in women and underserved populations.

- **Nancy Gardner Sewell Medicare Multi-Cancer Early Detection Screening Coverage Act (H.R.842):**
  Authorizes, beginning in 2028, Medicare coverage and payment for multi-cancer early detection screening tests that are approved by the FDA.

- **Scientific EXPERT Act (S.822):**
  Directs the FDA to actively support and participate in externally led, science-focused drug development meetings dedicated to rare diseases.

RDDC 2025 Legislative and Administrative Advocacy

RDDC led advocacy efforts to introduce the components of the Health Equity and Rare Disease (HEARD) Act (H.R.1750), ensuring that public and private nonprofits, health professional schools, and tribal and urban health centers are equipped to meaningfully engage, educate, and expand treatment access for rare disease communities. This effort also helps strengthen the pipeline of medical professionals trained to diagnose, treat and research rare diseases.

RDDC held the Resilient, Impassioned, Strong, and Empowered (RISE) Awards on National Rare Disease Day, recognizing exceptional leaders, advocates, and patients in the rare disease community, as well as bipartisan Members of Congress advancing critical health legislation. The event elevated national awareness of rare disease challenges and celebrated groundbreaking contributions across the ecosystem.

Hosted a session on Shaping Policy for Accessibility: Healthcare Policies Impacting Rare Disease Communities as part of the National Minority Quality Forum (NMQF) Leadership Summit to emphasize the importance of patient, provider, and stakeholder engagement in rare disease policy. The session highlighted disparities, showcased community-driven solutions, and underscored the need for sustained congressional attention.

RDDC hosted a Capitol Hill Briefing to bring together leaders from across sectors to address long standing disparities in rare disease diagnosis, treatment, and care. This unifying event amplifies the voices of underserved communities and champions equitable change in rare disease care.

Met with key congressional rare disease champions and stakeholder organizations to educate them about RDDC's policy priorities, advance shared goals, and begin shaping concrete plans for legislative and oversight actions in the coming year.

Convened an industry roundtable featuring a senior health staffer from the House Committee on Energy and Commerce, who provided insight into 2025 congressional dynamics and the future landscape for rare disease policy. Healthcare attorneys also briefed participants on the legal and tax implications of recent laws, helping industry and advocates prepare for upcoming regulatory and legislative developments.

Submitted public comments to FDA on its draft guidance regarding the inclusion of pregnant and breastfeeding women in clinical trials (E21), advocating for clearer pathways, stronger protections, and equitable research practices to ensure these populations are not systematically excluded from rare disease innovation.

Submitted public comments on the NIH Artificial Intelligence Strategic Plan urging the agency to design, use, and regulate AI tools with rare disease communities in mind, including improved data diversity, ethical safeguards, and innovation incentives that prioritize early and accurate diagnosis.

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