Legislative Priorities Reference Guide

The policy proposals listed in the Community Congress Legislative Priorities Reference Guide have been identified by the RARE Foundation and the Community Congress members as relevant to the rare disease community. Inclusion in this document does NOT serve as an endorsement of the proposals by the RARE Foundation.

LEAD Category: Represents the top priorities for RARE Foundation. This includes policies that RARE Foundation will craft strategy, conduct direct lobbying activities, activate advocates to support, and more.

Newborn Screening Federal Reauthorization & Modernization Efforts

The Need:
Newborn screening enables the early detection of disease and allows for critical interventions to begin at the earliest possible moment. In 2008, Congress passed the original Newborn Screening Saves Lives Act to facilitate comprehensive newborn screening in every state, and it was reauthorized in 2014. The authorization lapsed in 2019; however, the program is still funded through appropriations. The lapse in authorization inhibits the ability to make improvements to the programs as well as keeps them at risk of being shut down. In addition, any attempts to modernize the federal newborn screening system will require a new bill to make those changes.

The Proposed Solution:
The Newborn Screening Save Lives Reauthorization Act renews the federal programs that assist in states to improve and expand their newborn screening programs, supports parent and provider education, and ensures laboratory quality and surveillance for newborn screening. The reauthorization includes improvements such as increased transparency on the Recommended Uniform Screening Panel (RUSP) nomination process, grants to support pilot studies, and a National Academy of Medicine Study on how to modernize newborn screening.

The Status:
The Newborn Screening Saves Lives Reauthorization Act (H.R 4709) passed the House Energy & Commerce Health Subcommittee by voice vote in early September but was not included in a full Committee markup as language is still being finalized. Committee

staff and EveryLife, alongside coalition partners, are refining updated bill language based on community feedback, with no Senate companion bill introduced yet.

Contact: For more information on the status of the bill, please contact Dylan Simon at dsimon@rareadvocates.org.

Protect & Strengthen the Accelerated Approval Pathway

The Need:
The accelerated approval pathway allows the U.S. Food & Drug Administration (FDA) to use a surrogate endpoint (also called a biomarker) to evaluate the safety and efficacy of therapies for serious conditions with unmet needs. By evaluating a surrogate endpoint that is reasonably likely to predict clinical benefit, the pathway allows patients to access treatments at the earliest possible moment. The rare disease community deserves resources and innovative solutions that match the urgency and unmet need of the diseases with which we are diagnosed – and the staggering costs with which we are faced. Now is the time to realize the promise of accelerated approval to advance therapies for patients with rare diseases. Congress should encourage reforms that unleash this potential, not stymie it.

The Proposed Solution:
Several policies aimed at protecting and strengthening the accelerated approval pathway for the development of rare disease therapies were included in the 2022 Food and Drug Omnibus Reform Act. Despite these advancements, more work remains, including the need to protect against policies restricting coverage and reimbursement for therapies approved using the accelerated approval pathway. Examples of policies that would protect and strengthen the accelerated approval pathway include:

- Support the prioritization of resources and collaboration necessary to advance knowledge on rare disease biomarkers and intermediate clinical outcomes,
- While recognizing the regulatory authority of the FDA, facilitate earlier payer engagement prior to regulatory approval around the selection and use of endpoints and surrogate biomarkers in clinical studies and regulatory review,
- Identify opportunities for confirmatory trial process improvements through more structured and earlier stakeholder engagement in the pre-market setting, and
- Enable broader use of real-world evidence to satisfy post-approval confirmatory requirements.

•Prevent payers from restricting or denying coverage for a rare disease therapy solely because it was approved using the accelerated approval pathway.

The Status:
The RARE Foundation will monitor all legislation impacting accelerated approval and is actively exploring options to lead efforts to protect access to therapies that use the pathway.

Contact: For more information, please contact Jamie Sullivan at jsullivan@rareadvocates.org.

Orphan Drug Incentives

Rare Pediatric Disease PRV Reauthorization (Give Kids a Chance Act - H.R 1262 /S. 932)

The Need:
The Priority Review Voucher (PRV) program was expanded in 2012 to include drugs that treat rare pediatric diseases through the Food and Drug Administration Safety and Innovation Act (FDASIA).

After the FDA approves an eligible treatment, the company is issued a PRV. The opportunity to obtain a PRV is a significant incentive in pediatric rare disease therapy development, as a company that secures a PRV can utilize it for a future treatment that wouldn't otherwise qualify for priority review, resulting in approximately a 4-month reduction in review time. Some companies choose to sell the PRV to another company, generating revenue for the seller that is often used to continue and expand their rare disease research and development programs. The authorization for the Rare Pediatric Disease PRV Program expired on December 20, 2025.

The Proposed Solution:
The Give Kids a Chance Act of 2025 is a comprehensive bill designed to remove barriers in pediatric drug development and accelerate treatments for children in need. It incorporates provisions from four previous bills—the Give Kids a Chance Act, the Creating Hope Reauthorization Act, the Innovation in Pediatric Drugs Act, and the RARE Act—all of which failed to pass in the last Congress, despite bipartisan support and passage in the House. By incentivizing pediatric research through the renewal of the Rare Pediatric Disease PRV Program and strengthening pediatric drug laws, this legislation aims to improve access to life-saving therapies.

The Status:
The Give Kids a Chance Act of 2025 (H.R.1262 / S.932)—was introduced in the House by Representatives Michael McCaul (R-TX) and Debbie Dingell (D-MI) and in the Senate by Senators Markwayne Mullin (R-OK) and Michael Bennet (D-CO).

Following continued advocacy from the rare disease community, Congress ultimately included a five-year reauthorization of the Rare Pediatric Disease PRV Program in a broader bipartisan healthcare legislative package accompanying FY2026 appropriations. The legislation was passed by Congress in early 2026 and signed into law, restoring the program and enabling FDA to continue issuing vouchers for qualifying rare pediatric disease therapies.

Contact: For more information on the bill's status, please contact Jamie Sullivan at jsullivan@rareadvocates.org.

Orphan Drug Tax Credit/Cameron's Law (H.R. 1414)

The Need:
The Orphan Drug Act created a tax credit worth 50% of qualified research and development expenses for orphan-designated products. In the 2017 tax reform bill, the orphan drug tax credit was reduced from 50 percent to 25 percent, limiting the largest incentive for companies to invest in the development of therapies for rare diseases. In the years since 2017, proposals to further weaken the orphan drug tax credit have emerged.

The Proposed Solution:
Cameron's Law would reinstate the orphan drug tax credit to 50 percent. It also includes a CDC feasibility study of expanding the ability to track the epidemiology of rare diseases.

The Status:
Introduced in the House on March 3 by Representatives Josh Gottheimer (D-NJ-05) and Don Bacon (R-NE-02). It was referred to the Ways and Means and Energy and Commerce Committees for consideration.

Contact: For more information on the issue, please contact Jamie Sullivan at jsullivan@rareadvocates.org.

Appropriations

The Need:
The National Economic Burden of Rare Disease Study found that the annual burden of rare diseases was nearly $1 trillion in direct and indirect costs. The appropriations process allocates money to the government each fiscal year. Each fiscal year takes place from October 1st of one year to September 30th of the next.

The appropriations process starts with the President's Budget Request to Congress. Law requires this submission to occur by the first Monday of February, but it frequently arrives later. Then, Congress holds hearings and develops and considers a budget resolution, which provides a high-level plan that, in theory, should inform appropriations discussions. During this process, Congress hears testimony from the Executive branch to defend the President's initial recommendations. To complete the appropriations process, Congress must pass a series of 12 appropriations bills that fund the government every fiscal year. These bills cover one or multiple departments or agencies, such as:

- Labor, HHS, and Education: Funds CDC, NIH, and most other Health and Human Services agencies and the Departments of Labor and Education.
- Agriculture and FDA: Funds the Food and Drug Administration and Department of Agriculture.

The Proposed Solution:
The RARE Foundation has identified priorities for the Fiscal Year 2026 appropriations bills. While these priorities are currently in place, they may be updated or amended as the policy landscape evolves under the new administration.

- FDA's Rare Disease Innovation Hub and the Orphan Products Grant Program
- Federal Biomedical Research and Public Health Programs

The Status:
In March 2025, a Continuing Resolution (CR) was signed into law that will fund the government through September 30. The bill included cuts to vital programs like the Department of Defense's Congressionally Directed Medical Research Program and health agencies such as NIH and HRSA, while omitting key provisions like the reauthorization of the Rare Pediatric Disease PRV Program. Passing a CR through the end of the fiscal year also eliminates the opportunity to implement the report language included in the individual appropriations bills, such as the language for the Rare Disease Interagency Coordinating Committee.

The CR was the first item to be completed in three interconnected legislative processes—the CR, the FY2026 Appropriations Cycle, and Budget Reconciliation—that directly impact the rare disease community. The FY2026 appropriations cycle is poised to move quickly. Members of Congress seek input on community priorities from advocates. The Appropriations Committees develop the 12 pieces of legislation that will set funding levels and convey their priorities for federal agencies to address over the next year. This starts on October 1, 2025.

Contact: For more information on the RARE Foundation's appropriations priorities, please contact Dylan Simon at dsimon@rareadvocates.org.

ENGAGE Category: Represents policies on which The RARE Foundation will proactively act, including shaping legislative strategy alongside coalition partners, providing testimony, crafting comments, engaging with Congress, regulators, and state legislatures, and sharing opportunities to provide input with advocates.

BENEFIT Act

The Need:
Through the Prescription Drug User Fee Act reauthorization in 2012 and provisions in the 21st Century Cures Act in 2016, the FDA has programs and policies in place to ensure that patient perspectives are gathered and made available throughout the risk-benefit analysis of potential therapies. However, there is currently no requirement that the FDA use this data in their analysis or report on how the patient perspective factored into the risk-benefit analysis.

The Proposed Solution:
The Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act would amend the Food, Drug, and Cosmetic Act to ensure that patient experience, patient engagement, and patient-focused drug development data are included in the risk-benefit assessment. Additionally, the BENEFIT Act would require the FDA to report on how this data was used throughout the risk-benefit assessment.

The Status:
This bill has not yet been introduced in the 119th Congress. The lead sponsors in the House are engaging with advocates and the technical experts at the FDA to revise the bill text prior to introduction.

Contact: For more information on the BENEFIT Act, contact Lauren Stanford (lauren@parentprojectmd.org) with Parent Project Muscular Dystrophy.

The Scientific EXPERT Act (H.R 1532 / S. 822)

The Need:
Developing drugs for rare diseases is challenging due to limited patient populations, unclear regulatory pathways, and the need for specialized clinical trial designs. There is a need for structured discussions among experts to streamline the development of drugs for these conditions.

The Proposed Solution:
The Scientific External Process for Educated Review of Therapeutics (EXPERT) Act of 2025 The bill establishes a process for externally led science-focused drug development (EL-SFDD) meetings to address scientific and regulatory challenges in rare disease drug development. The Reagan-Udall Foundation will organize at least four annual meetings, bringing together medical experts, drug sponsors, patient organizations, and FDA representatives to discuss trial design, biomarkers, clinical endpoints, and other key issues. The outcomes will be documented to guide future drug development efforts.

The Status:
The bill was introduced in both chambers with the Rare Disease Congressional Caucus co-chairs as the original sponsors. In the House, Representatives Doris Matsui (D-CA-07) and Gus Bilirakis (R-FL-12) introduced the bill on February 24, 2025, and it was referred to the Committee on Energy and Commerce. In the Senate, Senators Amy Klobuchar (D-MN) and Roger Wicker (R-MS) reintroduced the bill.

Contact: For more information on the Scientific EXPERT Act, please contact Dylan Simon at dsimon@rareadvocates.org.

Medicaid Coverage

The Need:
Through traditional eligibility routes and waiver programs that enable coverage for children's complex health needs, Medicaid is a critical safeguard for our rare disease community. Changes to Medicaid will have direct impacts on our rare disease community.

The Solution:
The RARE Foundation will engage in efforts to defend Medicaid from major changes by educating members of Congress, collecting additional data to understand the impact of Medicaid on the rare disease community, and mobilizing advocates to share their personal experiences highlighting the importance of Medicaid.

The Status:
On February 25, the House passed its version of a budget resolution, which includes reconciliation instructions requiring $1.5 billion in spending cuts, including a $880 billion cut from the committee that oversees Medicare, Medicaid, and other health programs. The Senate must agree to the same framework, or it will pass its own version and seek

to work with the House on a compromise. If they do, then Congress will begin determining what spending cuts and other eligible policies to include.

Contact: For more information, please contact Jamie Sullivan at jsullivan@rareadvocates.org.

Genetic Testing Coverage

The Need:
Approximately 70% to 80% of rare diseases are genetic, making genetic testing critical for diagnosis. However, many patients, particularly those under 21, face significant barriers to accessing necessary tests. These barriers include inconsistent insurance coverage, limited testing options, and financial challenges, especially for Medicaid patients. As a result, delays in diagnosis and treatment can worsen health outcomes for pediatric or neonatal-onset genetic conditions. Expanding access to genetic testing is crucial for timely diagnoses and improved outcomes for these vulnerable populations. Genomic Answers for Children's Health Act (H.R 7118).

The Need:
Children with suspected rare genetic conditions often face lengthy diagnostic journeys, sometimes lasting years before receiving an accurate diagnosis. While advances in genomic sequencing—such as whole genome sequencing (WGS) and whole exome sequencing (WES)—have significantly improved the ability to identify rare diseases, coverage for these tests remains inconsistent, particularly within Medicaid.

Many children enrolled in Medicaid or living in rural and underserved communities experience delays in accessing comprehensive genomic testing due to unclear coverage policies and variation across states. These delays can prolong the diagnostic odyssey, lead to unnecessary testing and procedures, and delay appropriate care, treatment planning, and referrals. Improving access to advanced genetic diagnostics is critical to ensuring timely diagnosis and equitable care for children with suspected rare diseases.

The Proposed Solution:
The Genomic Answers for Children's Health Act seeks to improve access to advanced genomic testing for children by clarifying Medicaid coverage of whole genome sequencing (WGS) and whole exome sequencing (WES) under the Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) benefit.

The legislation would also direct the Secretary of Health and Human Services to convene national organizations and relevant stakeholders to identify best practices for improving access to genomic diagnostics and increasing awareness among providers and families. In addition, the bill requires a report to Congress evaluating access to testing, implementation challenges, and the impact of genomic diagnostics on patient care.

The Status:
The Genomic Answers for Children's Health Act (H.R. 7118) was introduced in the House of Representatives in early 2026 on a bipartisan basis.

Contact: For more information on the issue, please contact Dylan Simon at dsimon@rareadvocates.org.

SUPPORT Category: Represents policies led by existing coalitions or organizations. As bandwidth allows, the RARE Foundation's involvement is focused on amplifying efforts and adding the rare disease community's perspectives by joining sign-on letters, submitting testimony, engaging legislators, participating in joint meetings, or sharing opportunities to provide input with advocates.

Help Ensure Lower Patient (HELP) Copays Act (H.R. 6423 / S.864)

The Need:
Many patients with rare diseases and their families rely on various forms of co-pay assistance to afford their medications. Nationally, there has also been a trend toward higher deductibles, annual out-of-pocket maximums, and cost-sharing responsibilities for patients. Amidst these trends, pharmacy benefit managers (PBMs) and insurance companies are adding to access barriers by utilizing programs called co-pay accumulators that prevent any assistance received from counting toward patient cost-sharing responsibilities. These programs result in disruptions in care as co-pay assistance runs out and deductibles still aren't satisfied, leaving the patient on the hook for the full cost of medications.

The Proposed Solution:
The Help Ensure Lower Patient (HELP) Copays Act requires health plans to count the value of copay assistance toward patient cost-sharing requirements. The bill also closes a loophole in the Affordable Care Act that allows employer health plans to deem certain categories of prescription drugs as non-essential, thus not counting any cost-sharing towards the patient's deductible or out-of-pocket maximum.

The Status:
The HELP Copays Act was introduced in the Senate in March 2025 by Senators Roger Marshall (R-KS) and Tim Kaine (D-VA). Companion legislation was reintroduced in the House on December 4, 2025, by a bipartisan group of representatives, including Tom Kean, Jr. (R-NJ) and Nanette Barragán (D-CA). The bill has been referred to the House Committees on Energy and Commerce and Ways and Means and awaits further congressional consideration.

Contact: For more information, please contact the All Copays Count Coalition at info@allcopayscount.org.

Accelerating Kids' Access to Care Act (H.R. 1509 /S. 752)

The Need:
When children enrolled in Medicaid or CHIP (Children's Health Insurance Program) require access to medically necessary care out of state, there are often lengthy delays caused by the process of screening and enrolling out-of-state physicians as eligible providers in the child's home state's Medicaid or CHIP program. The process varies by state and requires providers to spend significant time completing the requirements.

The Proposed Solution:
The Accelerating Kids' Access to Care Act will establish a screening pathway that children's hospitals and related providers can utilize on a voluntary basis. If providers opt to use this pathway and are screened successfully, they can be enrolled in other state Medicaid programs if called upon to provide care to children in that state. Eligible providers will be limited to those providing care for children or, in limited cases, to people over 18 who are being treated for a condition that developed before age 18.

The legislation builds upon existing CMS activities, including an ongoing multi-state pilot in which CMS is screening providers via the Advanced Provider Screening System, and is also consistent with ongoing efforts to reduce regulatory burdens in healthcare. It is consistent with actions CMS has taken via the COVID-19 public health emergency (PHE) waivers to permit states to waive such requirements during the emergency.

Finally, the legislation pertains only to provider screening and enrollment and does not change the authority states have to authorize out-of-state care and negotiate payment with providers who accept such cases.

The Status:
The Accelerating Kids Access to Care Act (S.752/H.R.1509) was introduced on a bipartisan basis and was originally considered as part of a broader bipartisan healthcare policy package in 2024 but was not included in the final year-end funding bill.

Following continued advocacy from the rare disease and pediatric health communities, Congress ultimately included the Accelerating Kids Access to Care Act in a broader healthcare legislative package accompanying the FY2026 appropriations legislation. The bill passed Congress in early 2026 and was signed into law.

Contact: For more information or to get involved in supporting the Accelerating Kids Access to Care Act, please contact Nick Manetto at nicholas.manetto@faegredrinker.com.

PROTECT Rare Act (S. 3551)

The Need:
Ninety-five percent of rare diseases do not have an FDA-approved treatment, which results in many within the rare disease community relying on off-label use of drugs. However, those treatments are rarely covered by insurance, leading to high prescription drug costs.

The Proposed Solution:
The Providing Realistic Opportunity to Equal and Comparable Treatment for Rare (PROTECT) Act would allow Medicare and Medicaid to cover off-label use of FDA drugs based on reviewing "… peer-reviewed medical literature, clinical guidelines, or an expert in such disease or condition as identified by a medical society involved in the treatment or management of such disease or condition, and is not reviewed unfavorably in the compendia…" to determine if they should cover the drug for a rare disease. It will also require private payers to establish an expedited review pathway for formulary exceptions, reconsideration, and/or appeals of any denial of coverage for a drug or biological agent prescribed for a patient with a rare disorder.

The Status:
The PROTECT Rare Act was introduced in the Senate in 2025 as S.3551. The bill has been referred to the relevant Senate committee for consideration and awaits further congressional action.

Contact: For more information, please contact the Haystack Project at https://haystackproject.org/.

Medicaid VBPs for Patients (MVP) ACT (H.R. 7871 / S. 1637)

The Need:
Value-based purchasing (VBP) agreements base reimbursement for treatment on whether the drug works or not. They change the way drugs are reimbursed in the United States, but there are existing laws on the books that impede biopharmaceutical manufacturers from offering deep discounts to state Medicaid programs and private payers. To update these outdated laws, CMS issued a rule that aimed to eliminate many of the barriers. While this rule was a start, federal barriers remain and are preventing VBPs from succeeding in the marketplace.

The Proposed Solution:
The Medicaid VBPs for Patients (MVP) Act would remove federal barriers that prevent state Medicaid programs, private payers, and manufacturers from entering into value-based purchasing (VBP) agreements by codifying provisions from CMS's VBP Final Rule and adding VBPs to the list of safe harbors to the federal anti-kickback statute. The bill would also require a GAO study on the effectiveness of VBPs on patient access, health outcomes, and health system costs.

The Status:
The MVP Act was introduced in the Senate and referred to the relevant Senate committee for consideration. In the House, Congressman Brett Guthrie (R-KY-02), Chairman of the House Committee on Energy and Commerce, reintroduced the legislation alongside Representatives Jake Auchincloss (D-MA-04), John Joyce, M.D. (R-PA-13), Scott Peters (D-CA-50), Mariannette Miller-Meeks (R-IA-02), and Don Davis (D-NC-01).

Contact: For more information on the status of the bill please contact Sloan Salzburg at sloane.salzburg@cahc.net.

Joe Fiandra Access to Home Infusion Act (H.R. 4993)

The Need:
A 2020 proposed CMS rule would have clarified the Home Infusion Therapy Benefit in the 21st Century Cures Act to allow pump-administered drugs that require healthcare professional administration to be covered. Unfortunately, in 2021, CMS declined to finalize this policy, leaving some Medicare beneficiaries unable to receive infused treatments at home.

The Proposed Solution:
The Joe Fiandra Access to Home Infusion Act provides Medicare coverage for non-self-administered home infusion drugs when administered in a patient's home by a qualified professional. To be eligible, the treatment's labeling must require the drug to be prepared immediately before administration, administered by a qualified health professional, and required at least once per month.

The Status:
The Joe Fiandra Access to Home Infusion Act was included in a broader healthcare legislative package accompanying the FY2026 appropriations legislation. Congress passed the package in early 2026, and the provisions were signed into law.

Contact: To be connected to the individuals leading the bill effort, please contact Dylan Simon at dsimon@rareadvocates.org.

Safe Step Act (H.R. 5509 / S. 2903)

The Need:
Step therapy, also known as “fail first,” is a practice used by many health insurers that requires patients to try medications preferred by the insurer before being approved for the medication prescribed by a doctor. This can lead to a delay in treatment, along with severe and sometimes life-threatening disease progression.

The Proposed Solution:
This bill establishes an exception to an insurer’s step therapy process if (1) an otherwise required treatment has been ineffective, (2) such treatment is expected to be ineffective and delaying effective treatment would lead to irreversible consequences, (3) such treatment will cause or is likely to cause an adverse reaction to the individual, (4) such treatment is expected to prevent the individual from performing daily activities or occupational responsibilities, (5) the individual is stable based on the prescription drugs already selected, or (6) there are other circumstances as determined by the Employee Benefits Security Administration.

The bill requires a group health plan to implement and make a clear process available for an individual to request an exception to the protocol, including required information and criteria for granting an exception. The bill further specifies timelines under which plans must respond to such requests.

The Status:
The Safe Step Act (H.R. 5509 / S. 2903) was reintroduced in the 119th Congress (2025–2026) on a bipartisan basis. In the Senate, the legislation was introduced by Senator Lisa Murkowski (R-AK), and in the House by Representative Rick W. Allen (R-GA-12). The bill has been referred to the relevant committees of jurisdiction and awaits further congressional consideration.

Contact: For more information, please contact Jason Harris at jharris@psoriasis.org.

MINI Act (Maintaining Investments in New Innovation) (H.R 1672)

The Need:
Gene-targeted therapies (GTTs) hold significant potential for patients with rare diseases, offering life-changing or even curative treatments. Under the Inflation Reduction Act's Medicare Drug Price Negotiation Program rules, GTTs were classified as small molecules, resulting in a shorter period prior to becoming eligible for negotiation than large-molecule treatments that share more similarities. The 7-year exemption before becoming eligible for Medicare drug price negotiation creates financial uncertainty, making companies hesitant to invest in developing these treatments.

The Proposed Solution:
The Maintaining Investments in New Innovation (MINI) Act would align the timeline for GTTs' eligibility for Medicare negotiation with large molecule treatments. This additional time would encourage continued investment in rare disease therapies, increasing the likelihood of new and innovative treatments reaching patients.

The Status:
The Maintaining Investments in New Innovation (MINI) Act (H.R.1672) was introduced on February 27, 2025, in the 119th Congress (2025–2026) by Representative Don Davis (D-NC) with bipartisan support. The bill has been referred to by the House Committees on Energy and Commerce and Ways and Means and awaits further congressional action.

Contact: To be connected to the individuals leading the effort, please contact Jamie Sullivan at jsullivan@rareadvocates.org.

Supplemental Oxygen Access Reform (SOAR) Act (H.R. 2902 / S. 1406)

The Need:
Patients who rely on supplemental oxygen, including those with rare respiratory conditions, often face challenges accessing necessary equipment and services due to Medicare's competitive bidding system. Limited supplier options and inconsistent reimbursement policies create barriers to quality care and continuity of treatment.

The Proposed Solution:
The Supplemental Oxygen Access Reform (SOAR) Act reforms Medicare's payment structure by providing separate, inflation-indexed payments for oxygen equipment, supplies, and services. It ensures that respiratory therapists' services are specifically covered and appropriately reimbursed. Additionally, the bill mandates the development

of an electronic prescription template to streamline access and strengthen patient rights, including supplier choice and transparent communication.

The Status:
The Supplemental Oxygen Access Reform (SOAR) Act (H.R. 2902 / S. 1406) has been introduced on a bipartisan basis in both chambers of Congress. In the House, the bill was introduced by Representative David Valadao (R-CA) along with bipartisan cosponsors, and in the Senate by Senator Bill Cassidy (R-LA) with bipartisan support. The legislation has been referred to the House Committees on Energy and Commerce and Ways and Means, and the Senate Committee on Finance, and awaits further congressional consideration.

Contact: To be connected to the individuals leading the effort, please contact Jamie Sullivan at jsullivan@rareadvocates.org

Ensuring Nationwide Access to Better Life Experience (ENABLE) Act (H.R. 1436/ S. 627)

The Need:
ABLE accounts provide individuals with disabilities a critical means to save for essential expenses, such as housing, transportation, and assistive technology, without losing eligibility for federal benefits like Medicaid and Supplemental Security Income. Key tax provisions supporting ABLE account holders are set to expire in 2025, creating uncertainty for individuals and families relying on these financial tools.

The Proposed Solution:
The Ensuring Nationwide Access to Better Life Experience (ENABLE) Act would make three key tax provisions permanent: (1) ABLE to Work, allowing additional contributions for employed individuals with disabilities, (2) the ABLE Saver's Credit, providing a tax credit for contributions to ABLE accounts, and (3) 529-to-ABLE rollovers, enabling funds from education savings accounts to be transferred into ABLE accounts tax-free. These changes would enhance long-term financial stability for people with disabilities.

The Status:
The Ensuring Nationwide Access to Better Life Experience (ENABLE) Act (H.R.1436 / S.627) was introduced on a bipartisan basis in the 119th Congress by Representative Lloyd Smucker (R-PA) and Representative Don Beyer (D-VA) in the House, with companion legislation introduced in the Senate. Following continued bipartisan support for strengthening financial tools for individuals with disabilities, provisions from the

ENABLE Act were ultimately included in a broader legislative package enacted in 2026. The legislation permanently extends key tax provisions supporting ABLE accounts that were previously scheduled to expire in 2025.

Contact: To be connected with the individuals leading the effort, please contact Dylan Simon at dsimon@rareadvocates.org.

The Access to Genetic Counselor Services Act (H.R. 6280 / S. 3607)

The Need:
Genetic counselors play a critical role in helping patients and families understand genetic conditions, interpret complex test results, and guide medical decision-making for hereditary diseases and cancer. However, genetic counselors are not currently recognized as independent providers under Medicare, limiting patient access to their services. This gap can result in longer wait times for genetic testing, delayed diagnoses, and missed opportunities to inform care decisions and reduce unnecessary testing.

The Proposed Solution:
The Access to Genetic Counselor Services Act would amend the Social Security Act to recognize licensed or certified genetic counselors as Medicare providers, allowing them to bill Medicare directly for services under Medicare Part B. Services would be reimbursed at 85 percent of the physician fee schedule, consistent with other non-physician providers. The legislation defines qualified genetic counselors as individuals licensed by their state or certified by the American Board of Genetic Counseling (ABGC) in states without licensure. Expanding provider recognition would improve patient access to genetic counseling, support more informed care decisions, and help reduce unnecessary healthcare costs.

The Status:
The Access to Genetic Counselor Services Act (H.R. 6280 / S. 3607) was introduced in the 119th Congress (2025–2026) on a bipartisan basis by Representatives Adrian Smith (R-NE) and Kathy Castor (D-FL) and Senators John Barrasso (R-WY) and Peter Welch (D-VT). The legislation has been referred to the relevant committees of jurisdiction and awaits further congressional consideration.

Contact: For more information on the Access to Genetic Counselor Services Act or status of the Senate bill please contact Dylan Simon at dsimon@rareadvoates.org.

ACA Enhanced Premium Tax Credits (H.R. 5145)

The Need:
Enhanced Affordable Care Act (ACA) premium tax credits were expanded through the American Rescue Plan Act (ARPA) and later extended by the Inflation Reduction Act (IRA), significantly lowering marketplace premiums for millions of Americans. These enhanced subsidies expired on December 31, 2025, reverting eligibility and subsidy levels to pre-2021 rules.

Without the enhanced credits, many individuals and families face higher premiums and may lose coverage. For rare disease patients who rely on marketplace plans when employer or public coverage is unavailable, affordability and continuity of coverage are critical to maintaining access to specialized care, diagnostics, and treatments.

The Proposed Solution:
The Bipartisan Premium Tax Credit Extension Act (H.R. 5145) extends the enhanced ACA premium tax credits that expanded eligibility and increased subsidy levels for marketplace coverage. The legislation seeks to preserve affordability for individuals and families purchasing coverage through the ACA marketplaces and help extend coverage disruptions that could limit access to care.

The Status:
The Bipartisan Premium Tax Credit Extension Act (H.R. 5145) was introduced in September 2025 and referred to the House Committee on Ways and Means. In January 2026, the House passed legislation extending the enhanced premium tax credits and sent the measure to the Senate for consideration. The Senate has not yet passed an extension, and negotiations continue as lawmakers consider competing proposals. Congress has not yet enacted a final extension, leaving the issue unresolved as discussions continue during the 119th Congress.

Contact: For more information on the bill, please contact Dylan Simon at dsimon@rareadvoates.org.

Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) Reauthorization (H.R. 5100/ S. 3971)

The Need:
The Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs provide critical federal funding to support early-stage

innovation and technology development. These programs have played an important role in advancing biomedical research, including rare disease therapies, diagnostics, and medical devices. Between 1996 and 2020, more than 100 FDA-approved drugs and thousands of medical devices were supported by SBIR/STTR awards. Without timely congressional action to extend these programs, funding disruptions could slow innovation and hinder small biotechnology companies working to develop treatments for rare diseases.

The Proposed Solution:
H.R. 5100 would extend the SBIR and STTR programs for one year, allowing the programs to continue operating while Congress negotiates a longer-term reauthorization. The extension aims to ensure continuity of funding for small businesses, startups, and research institutions developing new technologies and therapies.

The Status:
H.R. 5100 passed the House of Representatives in September 2025 under suspension of the rules, and the Senate companion, S. 3971, was signed into law on April 13, 2026. The legislation reauthorizes the Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs through September 30, 2031. It includes reforms to strengthen program integrity, safeguard against foreign adversaries, expand access for emerging innovative businesses, and enhance accountability to ensure federal investments deliver measurable results. These updates are intended to modernize program operations and sustain U.S. innovation, including continued support for early-stage research and development.

Contact: For more information on the bill, please contact Dylan Simon at dsimon@rareadvoates.org

MONITOR Category: Represents legislation that includes issues relevant to the rare disease community that should be followed for updates that necessitate further consideration.

Clinical Trial Diversity Policies

The Need:
Diverse communities are often underrepresented in clinical trials. Diverse clinical trials can lead to better health outcomes and ensure that products are designed for and effective for all communities. Several barriers to clinical trial participation may deter diverse populations from participating. Some of these barriers include geographic location, financial status, and educational barriers. To progress scientific development, it is essential to include individuals with varied living experiences and conditions. Clinical trial diversity helps to ensure that drugs, products, and devices are safe and effective for every individual.

The Proposed Solution:
The 2022 Food and Drug Omnibus Reform Act (FDORA) required the FDA to finalize guidance by June 2025 to improve diversity in clinical trials. The goal is to ensure trial populations better represent the demographics of those who will use the treatments.

The Status:
FDORA included key provisions to enhance clinical trial diversity, but additional policy efforts are still needed. FDA has released a Draft Guidance for Industry – Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies.

Other legislative proposals are under consideration, and the RARE Foundation will continue to monitor and advocate for further progress.

Drug Pricing Policies

The Need:
Over 30 million Americans live with one or more rare diseases, facing substantial financial burdens from medical and non-medical expenses. The 2019 National Economic Burden of Rare Disease Study estimated that the total annual economic impact of rare diseases exceeded $966 billion, including $437 billion in productivity losses, $418 billion in direct medical costs, and $111 billion in non-medical expenses.

Despite scientific advancements, 95% of rare diseases still lack an FDA-approved treatment. The clinical trial process for rare disease therapies is more complex, taking twice as long as non-orphan drugs and carrying higher failure risks.

As policymakers consider drug pricing reforms, it is critical to ensure that efforts to improve affordability do not inadvertently hinder innovation or restrict access to life-saving treatments. Price control policies in other countries have led to delayed access to new therapies—delays that rare disease patients in the U.S. cannot afford.

The Proposed Solution:
Community Congress maintains a drug pricing statement that focuses on preserving incentives for rare disease drug development while ensuring patient access to life-saving therapies. Read our full statement here.

The Status:
Each Congress, numerous bills focused on lowering drug costs are introduced. The RARE Foundation will monitor proposed policies and evaluate new evidence regarding the impact of proposed and current policies on rare disease therapy development and patient access.

Telehealth & Data Security Policies

The Need:
Telehealth has become a vital tool for patients with rare diseases who frequently encounter significant barriers to accessing specialist care. It improves access to expert care, reduces travel burdens, and facilitates participation in clinical trials. However, gaps in insurance coverage, inconsistent state policies, and regulatory restrictions limit its full potential. Additionally, secure data sharing is essential for advancing rare disease research and personalized treatments, but concerns over privacy, Integration, and patient control of data must be addressed.

The Proposed Solution:

- Expand Telehealth Access – Ensure permanent reimbursement for telehealth services across all insurance providers, including Medicare and Medicaid, to maintain accessibility for patients with rare diseases.
- Harmonize State and Federal Policies – Standardize telehealth regulations to allow cross-state provider access and facilitate specialist consultations.

•Enhance Data Privacy & Integration – Develop policies that protect patient data while enabling secure and efficient data sharing for research and treatment advancements.

•Support Digital Health Innovation – Encourage the integration of remote monitoring, wearable technology, and AI-driven tools to improve disease management.

The Status:
While pandemic-era telehealth flexibilities have improved access, many policies remain temporary or vary by state. Advocacy efforts continue to push for permanent telehealth coverage, expanded interstate provider access, and stronger data security protections. Policymakers are also exploring strategies to balance patient privacy with the need for robust frameworks that facilitate the sharing of data on rare diseases. Ongoing discussions focus on sustaining digital health innovations while ensuring equitable access for all patients with rare diseases.

Prior Authorization Reform

The Need:
Utilization management techniques, such as prior authorization, create significant barriers to accessing life-saving treatments, especially for individuals with rare diseases. A 2020 study of Medicare Part D formularies found that 76% of orphan drugs were subject to prior authorization, resulting in delays, negative clinical outcomes, and increased hospitalizations. While 94% of prior authorization requests were approved in 2021, over 2 million requests were denied, with only 11% of denials being appealed. Even when appeals were made, they had an 80% success rate, further illustrating the inefficiencies and obstacles in the current system. Rare disease patients often face prolonged waiting periods and increased uncertainty, delaying access to therapies that are crucial for their health.

The Proposed Solution:
Comprehensive prior authorization reforms are needed to streamline and improve the process for rare disease patients. Several proposals at both the state and federal levels aim to reduce delays and improve access. Reforms could include mechanisms that bypass certain prior authorization requirements, particularly for providers with a history of high approval rates.

Additionally, reforms have focused on electronic prior authorization system requirements, shorter response timelines, more transparency, and greater roles for

clinical experts. The goal is to create a more patient-centered system, reducing unnecessary delays and ensuring quicker access to life-saving therapies.

The Status:
While specific bills, such as the GOLD Card Act and the Improving Seniors' Access to Timely Care Act, have proposed solutions for reforming prior authorization, these pieces of legislation have not yet been reintroduced. Advocacy efforts continue to push for these reforms, aiming to reduce the administrative burden on healthcare providers and patients while ensuring quicker, more consistent access to treatments.

Further legislative work is expected to focus on ensuring that prior authorization processes support timely and equitable access for patients with rare diseases.

Laboratory Developed Test Regulation

The Need:
Diagnostic tests are currently regulated by two separate agencies: the FDA, which oversees in vitro diagnostics (IVDs) as medical devices, and CMS, which regulates a subset of IVDs known as laboratory-developed tests (LDTs). LDTs, often referred to as "home brew" tests, are developed and used within a single laboratory.

As LDTs continue to grow in complexity, the regulatory framework becomes fragmented, with different tests subjected to varying standards and approval processes. This lack of uniformity in regulation can lead to inconsistencies in test quality, access, and patient safety, particularly for those with rare diseases.

The Proposed Solution:
Our proposed solution for LDT regulation focuses on protecting rare disease innovation and ensuring continued access to essential diagnostic tests, including newborn screening. We advocate for policies that minimize regulatory burdens on small laboratories, provide extended transition periods, and exempt rare disease diagnostics from excessive fees. Safeguarding LDTs is critical to supporting early diagnosis, advancing clinical research, and preserving access to life-saving tests. Read our full statement here.

The Status:
The FDA's final rule on Laboratory Developed Tests (LDTs) took effect on May 6, 2024, confirming that in vitro diagnostics (IVDs), including LDTs, are regulated as medical devices under the Federal Food, Drug, and Cosmetic Act. The rule phases out

enforcement discretion over four years, requiring most LDTs to comply with FDA regulations, including premarket review and quality standards.

The Verifying Accurate Leading-edge IVCT Development (VALID) Act, which proposed an alternative regulatory framework for in vitro clinical tests (IVCTs), has not been reintroduced this session and is not actively promoted by all stakeholders. The FDA's rule remains the primary regulatory directive for LDTs.

PBM Reform

The Need:
Pharmacy Benefit Managers (PBMs) manage prescription drug benefits on behalf of public and private insurers. They manage formularies, negotiate rebates from drug manufacturers, and work with pharmacies to reimburse drugs dispensed to beneficiaries. PBMs claim to have helped to slow the growth of drug spending in recent years, but they are often incentivized to utilize high-priced drugs over cost-effective drugs based on how rebates are calculated. Over the last few years, concerns over lack of transparency as well as PBM's role in rising drug prices have led to Congress investigating policies to address issues within the PBM industry.

The Proposed Solution:
Policymakers are generally considering three reform options: increased transparency for rebates, requiring PBMs to pass through rebates to payers or patients, and banning spread pricing. Multiple bills have been introduced that would address a variety of policy changes.

The Status:
The RARE Foundation will monitor PBM reform proposals as they emerge and advance in the House and Senate.

QALY Bans

The Need:
QALYs (quality-adjusted life years) and similar metrics are often used to assess cost-effectiveness and inform reimbursement decisions, but they are widely considered inappropriate for evaluating the value of treatments for rare diseases.

As noted in the Rare Disease Community Statement on Drug Pricing Policies, these measures fail to account for the full impact of rare diseases, such as the significant burden they place on patients. Current frameworks often overlook patient perspectives and scientific factors that are crucial for evaluating treatments for rare conditions. Previous legislation has already imposed restrictions on the use of QALYs for Medicare, the restrictions do not apply across all federal healthcare programs.

The Proposed Solution:
The Protecting Health Care for All Patients Act proposed to prohibit the use of QALY or similar metrics in making coverage, formulary, or reimbursement decisions across all federal healthcare programs. This would ensure that the value of rare disease treatments is assessed in a way that better reflects the needs and experiences of patients without being constrained by discriminatory or inadequate measures.

The Status:
The bill has not yet been introduced in the 119th Congress.

NIH Reform

The Need:
The National Institutes of Health (NIH) plays a crucial role in funding and conducting biomedical research, including studies on rare diseases. The NIH includes 27 Institutes and Centers. Most rare disease communities are served by multiple ICs. The National Center for Advancing Translational Sciences (NCATS) is considered the ‘rare disease home’ at NIH.

The Proposed Solution:
There are opportunities to improve and build upon our current federal commitment to the vital role the NIH plays in our biomedical ecosystem and in protecting and advancing the health out-comes of all Americans. If Congress explores reforms to the NIH, the desired change can only be achieved through a transparent, deliberative, and inclusive process.

The Status:
In the 118th Congress, two proposals to reorganize the NIH and revise policies were released for comment. The RARE Foundation continues to monitor any changes made to the NIH through executive orders or proposed legislation in Congress to determine what action is necessary.

Alternative Funding Programs

The Need:
Access to specialty medications, crucial for treating rare, complex, and chronic conditions, is being hindered by the growing use of Alternative Funding Programs (AFPs).

AFPs are cost-saving strategies used by employer-sponsored, self-funded health insurance plans to minimize expenses.

AFPs typically carve out specialty medication coverage from the employer's benefit design, requiring patients to utilize available charitable assistance funds or other sources to access the medication.

AFPs are designed to save money for employers but can result in significant delays in accessing necessary medications, force patients to pay full price for medications that are not covered, and increase out-of-pocket costs, as they don't count toward deductibles or annual cost-sharing limits.

AFPs also raise concerns about the safety and efficacy of medications sourced through international importation programs.

The Proposed Solution:
Patient advocacy organizations and other stakeholders have begun to develop legislative, legal, and regulatory policy solutions to prevent AFP's harmful impacts on patient access. You can view the latest information about AFPs here. One legislative strategy being considered would prohibit health plans from changing or conditioning the terms of coverage based on the availability of financial or other product assistance for a drug.

The Status:
Federal legislation has not yet been introduced in the 119th Congress.