Rare Disease
Company Coalition

March 10, 2025

The Honorable Robert F. Kennedy Jr.
Secretary, U.S. Department of Health and Human Services
200 Independence Ave, SW
Washington, DC 20201

Dear Secretary Kennedy:

On behalf of the Rare Disease Company Coalition (RDCC) and the 29 rare disease companies we represent, we are proud to present our critical policy priorities for the year ahead. We are thankful for your commitment to spurring biomedical innovation and we look forward to working with your department on the policies that directly impact the millions of American families affected by rare diseases.

RDCC members are dedicated to discovering, developing, and delivering treatments for patients living with a rare disease. Collectively, RDCC members invested more than $17 billion in research & development (R&D) in 2023 and continue to reinvest over half of annual expenditures back into R&D. Our members have brought over 50 rare disease treatments to market and have more than 200 promising programs currently in development, underscoring our unwavering commitment to transforming patients' lives.

While rare diseases are defined by small patient populations – ranging from 1 to 200,000 patients in the United States – they are anything but rare. One in ten Americans live with a rare disease, and these diseases have an outsized impact on our nation's children. 1 in 2 patients diagnosed with a rare disease are children, and a third of these children do not live to see their 5th birthday. However, developing and delivering treatments for rare diseases is uniquely complicated, costly, and time-consuming. Challenges include small patient populations, complex and variable diseases with limited natural history, slow disease progression with often irreversible symptoms, and a lack of defined endpoints and biomarkers.

Beginning under the Reagan administration, innovation in rare disease therapies has surged over the past four decades. Prior to the enactment of the Orphan Drug Act in 1983, there were only 38 therapies for rare diseases on the market. Today, there are over 650 treatments available. Pro-innovation policies have driven this expansion and provided the rare disease ecosystem with the incentives necessary to support continued development.

Significant progress has been made in addressing the needs of people with rare diseases, but these advancements are meaningless if patients are unable to access them. Medicaid plays a crucial role in providing coverage for nearly half of all children with special health care needs, including rare diseases. Retaining access to Medicaid is essential to ensure these children can

benefit from groundbreaking treatments and innovations. Further, it is crucial that coverage and reimbursement policies support timely access to treatment for Americans with rare diseases.

Our policy priorities are driven by the needs of our community and a shared commitment to ensuring that the roughly 30 million Americans affected by one of the 10,000 rare diseases have access to life-changing treatments. As a coalition, we are united by an urgent responsibility to advocate for the millions of American patients and families affected by a rare disease.[1]

The RDCC offers the following recommendations to support the Trump Administration's commitment to increasing affordability, increasing efficiency, and improving the U.S. healthcare system:

1. Protect the Rare Disease Incentive Ecosystem
2. Support a Robust Regulatory System to Enable New Innovations
3. Ensure Efficient and Predictable Patient Access to Rare Disease Treatments

Protect the Rare Disease Incentive Ecosystem

Rare disease companies face unique risks in the race to discover, develop, and deliver treatments. With high research and development (R&D) costs stretching into the millions and billions, and a long, challenging journey from development to market, rare disease companies rely heavily on private investment and product revenue to survive. Policy and regulatory decisions can strain an already-difficult investment thesis and threaten critical capital investments. These decisions ultimately make or break the ability for rare disease companies to deliver hope for the over 30 million Americans living with a rare disease.

Despite these challenges, rare disease companies remain industry leaders in R&D investment. Both clinical and commercial stage rare disease companies invest over twice as much in R&D as their non-rare counterparts. And while only a third of commercial-stage rare disease companies achieve profitability, these companies invest nearly a third of their annual revenue back into R&D.[2]

However, investors are listening— and recent policy and regulatory changes have substantially reduced investments in rare disease companies. The trading index for rare disease companies has declined by nearly 7% per year over the last 5 years, compared to a 1.3% decline for non-rare disease companies. Rare disease companies saw nearly $10 billion less in investment available for research in 2022, stemming from decreases in venture capital investments, the initial public offering (IPO) market, and partnership revenues. The decrease in private investment poses a

[1] https://www.nih.gov/about-nih/what-we-do/nih-turning-discovery-into-health/promise-precision-medicine/rare-diseases

[2] Health Capital Group, Rare Disease Companies in the Public Markets: Challenging Performance Against a Backdrop of Policy Uncertainty. October 2023.

serious threat to progress in rare disease drug development, as the private sector contributes $160 billion annually —making up nearly 98% of total R&D funding.[3]

Fortunately, there are proven policies that help de-risk innovation for pediatric rare disease programs and increase the availability of treatments for children living with rare diseases.

Restore the Rare Pediatric Disease Priority Review Voucher (PRV) Program
The rare pediatric disease priority review voucher (PRV) Program, established in 2012 and reauthorized with robust bipartisan support in 2016 and 2020, is a no-cost, budget-neutral program designed to encourage the development of treatments for rare pediatric diseases, which are associated with urgent, unmet medical need. It has spurred significant innovation, leading to new therapies that have positively impacted over 200,000 patients across 47 rare pediatric indications. Moreover, more than 90 percent of all priority review vouchers were awarded to therapies for indications with no approved therapy on the market.[4] Despite its profound impact over the past 13 years, the true impact of the program is expanding and still being realized, as more than half of all PRVs were granted in the last four years alone.

The rare pediatric disease PRV program is a proven tool for spurring R&D investment for rare pediatric diseases and can help attract critical funding for small, emerging biopharmaceutical companies. However, the program began to lapse at the end of 2024. Without urgent restoration, ongoing development efforts will be jeopardized and future investment in rare disease programs will be destabilized. We urge your department and the Trump Administration to support Congressional efforts to reauthorize the rare pediatric disease PRV program through September 30, 2029.

Restore and Protect the Orphan Drug Tax Credit (ODTC)
The Orphan Drug Tax Credit is an integral provision within the Orphan Drug Act of 1983. The ODTC helps offset the cost of developing and testing rare disease therapies as they move through the clinical trial process. This long-standing incentive is particularly important for the many smaller companies focused exclusively on rare diseases. ODTC is available to drug sponsors that meet the IRS four-part test criteria for R&D and the Orphan Drug Designation criteria and allows them to offset qualifying R&D costs.

The ODTC is a vital incentive for companies working to bring rare disease treatments to market, as it reduced the significant financial cost and risk that comes with the journey from bench to bedside, which costs millions to billions of dollars per product and can take up to 15 years. Given its role in spurring rare disease R&D that may not otherwise be feasible, the ODTC is a lifeline to patients.

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[3] Ibid.
[4] Rare Disease Company Coalition, "Impact of the Priority Review Voucher Program on Rare Pediatric Disease Drug Development." May 2024.

Further reduction of the ODTC would have a devastating impact on rare disease drug development in the United States and on the millions of Americans living with a rare disease. We urge the Administration to support the preservation and restoration of the ODTC to its original 50 percent.

Support a Robust Regulatory System to Enable New Innovations

With 95 percent of rare diseases lacking a Food and Drug Administration (FDA)-approved treatment, there remains a significant and unmet medical need for first-in-class and best-in-class therapies. RDCC strongly supports approaches to ensure that the Food and Drug Administration (FDA) is positioned and resourced to exercise its scientific and regulatory leadership to meet the unique challenges and needs of rare disease drug development.

Preserve and Enhance FDA's Leadership and Expertise to Maintain the U.S. as the Global Leader in Biomedical Innovation

The United States has long been at the forefront of biopharmaceutical innovation, driving advancements in medical treatments and therapies that improve the lives of people with rare diseases worldwide. This leadership is supported by a strong, effective regulatory system that represents the global gold standard. The FDA's deep expertise ensures that treatments are safe, effective, and accessible to Americans who depend on them. Visionary FDA leaders are critical to advancing innovative, efficient approaches to regulation and ensuring these approaches are implemented consistently in product reviews. It is crucial to preserve and enhance the FDA's capacity to keep pace with current science. The RDCC advocates for a robust FDA with strong leadership and qualified and expert review teams so that our nation can continue to lead in biomedical innovation and deliver life-changing therapies to improve the health of Americans across the U.S.

Encourage FDA to Leverage All Relevant Authorities to Ensure that Safe and Effective Treatments are Available in a Timely Manner

For rare disease drug development, one size does not fit all. Rare diseases involve unique challenges, such as small, heterogeneous patient populations; diagnostic delays; and slow rates of progression for some diseases. Over the past several decades, Congress has enacted laws and FDA has issued regulations and policies that together comprise a rare disease "toolkit." However, there is an opportunity to ensure that the FDA's regulatory tools are more consistently applied. In 2023, the Congressional Rare Disease Caucus urged FDA to "improve policy and uniformity across the Agency for the review of rare disease therapy and product applications" citing "significant, uneven application of rare disease policies, guidance, and expertise, even, at times, for the same product application.[5]" The Rare Disease Company Coalition encourages the FDA to fully exercise its existing authorities throughout the development and review of rare

[5] Letter from Rare Disease Congressional Caucus to FDA Commissioner Robert Califf. May 2023. https://www.klobuchar.senate.gov/public/_cache/files/8/6/86568676-ebfc-4416-a714-27a249fa34a2/B40F79902D4A9B717A210312961E17CA.05032023-letter.pdf

disease treatments to ensure that safe and effective rare disease products are available to patients in a timely manner.

Ensure Efficient and Predictable Patient Access to Rare Disease Treatments

Even the most innovative and effective rare disease treatments are only as valuable as they are accessible. For rare disease patients who are fortunate to have an FDA-approved treatment for their condition, we must advance policies that facilitate timely access so patients can benefit from treatments as soon as they are available.

Expand the Inflation Reduction Act's Orphan Drug Exemption

The Inflation Reduction Act's (IRA) Orphan Drug Exemption (ODE) offers a price negotiation exemption for orphan drugs that treat only one orphan condition, thereby discouraging rare disease companies from further exploring promising research that could lead to additional treatment options. In practice, however, 1 in 5 orphan drugs are FDA-approved for more than one use, and 60 percent of those second indications are for another rare disease.[6]

The current limits imposed by the IRA that restrict the ODE to a single rare disease have created a roadblock to innovation, and in turn, progress towards developing treatments for more than 95 percent of rare diseases without approved therapies. Expanding the ODE to include multiple rare disease indications will remove unnecessary hurdles towards developing treatments for individuals with rare diseases. RDCC member companies understand the importance of investing back into research and development for rare diseases therapies, but to maximize the pursuit of promising research, we must expand the IRA's single-orphan exclusion.

Ensure Coverage of Rare Disease Treatments Through Public and Private Payers

Policy must ensure that both public and private payers uphold the FDA's statutory authority—including for drugs approved through expedited development and review programs—in determining a medical product's safety and efficacy. This includes preventing payers from adopting pricing and reimbursement policies that would disincentivize the use of FDA's expedited development and review programs, and other regulatory tools to overcome barriers to rare disease drug development and speed patient access.

First, Medicaid is a critical resource for Americans living with a rare disease and coverage decisions have significant consequences for timely access. Restrictive policies and barriers to coverage impose disproportionate and often insurmountable burdens on vulnerable patient populations and their families, who may have no other path to diagnosis and treatment. The Centers for Medicare & Medicaid Services (CMS) should be encouraged to promote timely patient access to treatment and to achieve shorter, streamlined drug coverage approval processes. Additionally, rare disease patients often see providers in multiple states as there may be a limited number of specialists/experts for their given disease or condition. Yet, rules for credentialing

[6] Health Capital Group, "Rare Disease Companies in the Public Markets: Challenging Performance Against a Backdrop of Policy Uncertainty." October 2023.

providers are complex and disincentivize coverage for out-of-state providers. This results in a patchwork system that discourages patient access to care. Policymakers should streamline the complicated and lengthy Medicaid provider screening and enrollment process and simplify access to out-of-state coverage.

Second, the accelerated approval pathway (AAP) is a critical tool that can address the unique challenges of rare disease drug development and provide rare disease patients with earlier access to treatment based on initial evidence of safety and effectiveness, while confirmatory studies required to verify clinical benefit(s) are ongoing. However, some federal proposals have shown a willingness by public and private payers to modify coverage protocols based on arbitrary conditions of a drug's effectiveness in certain populations, which would limit patient access to life-saving treatments. A recent study found these types of changes to accelerated approval would put therapies that have the potential to address the needs of 3.6 million patients at risk of withdrawal.[7] Any future policy reforms to the AAP should protect patient access, build upon Congress' recent steps to boost FDA oversight of accelerated approval products, leverage and enhance its existing authorities, and protect the integrity of this critical pathway.

Conclusion

Thank you for acknowledging the 30 million Americans and their families who are affected by a rare disease. We are grateful for your consideration of our collective policy priorities as you and your team work to enhance the nation's health and well-being throughout your department and the President Trump's Administration. Our organization is ready to serve as a resource and would welcome the opportunity to meet and discuss the priorities of the rare disease community. If you have any questions or would like to reach out further, please contact Stacey Frisk, Executive Director, at stacey@rarecoalition.com.

Sincerely,

Stacey Frisk
Executive Director
Rare Disease Company Coalition

[7] Vital Transformation, "Calculating the Value and Impact of Accelerated Approvals: Preliminary Findings." June 2022