Rare Disease
Company Coalition

# Arbitrary Price Controls Have a Negative, Disproportionate Impact on Rare Disease Treatment Development

## Preserving Existing Programs are Necessary for Continued Investment and Innovation

Policymakers must recognize that draconian measures like government-dictated drug pricing would disproportionately impact people living with rare diseases by defunding research and development for the most challenging diseases which Congress has worked so hard to incentivize, and by limiting patient access to innovative therapies. As rare disease companies dedicated to bringing treatment options to individuals with limited to no options, we advocate for better informed policies that account for the challenges of rare disease research and development and preserve the incentives required for continued investment and innovation.

## The Medical Community has Faced Severe Challenges in Researching and Understanding Rare Diseases

Rare diseases have historically - and sadly - attracted minimal attention because of the significant financial challenges of attracting capital to develop therapies for small patient populations. Although rare diseases affect more people than AIDS and cancer combined, more than 93 percent of rare diseases do not have an FDA-approved treatment. For decades, and often with barely any financial backing, the medical community has faced severe challenges in researching and understanding rare diseases.

## Rare Disease Policy Has Changed the Future for People Living with Rare Diseases; Progress is Threatened by Discussions Permitting the Government to Dictate the Launch Prices of Rare Disease Therapies

Over the last several decades, rare disease companies have been able to address a growing number of unmet needs for people living with rare diseases that were once untreatable. Since implementation of the *Orphan Drug Act* in 1983, there have been more than 1,000 FDA approvals for rare disease treatments, with over 25 percent of those approvals occurring in the last three years.

Prior to enactment of the *Orphan Drug Act*, only ten therapies were available to treat rare diseases. It took decades after the passage of the *Orphan Drug Act* to see meaningful results for rare disease patients. We must preserve this innovation.

Rare disease policy encapsulated in the *Orphan Drug Act*, and subsequently by the *Food and Drug Administration Safety and Innovation Act* and the *21st Century Cures Act*, has changed the future for people living with rare diseases by incentivizing innovation. These purposeful acts of Congress have allowed the rare disease research community to capture the urgency, creativity, and adaptability needed for the development of rare disease therapies.

This progress for people living with rare diseases is threatened by current discussions that would permit the government to dictate the launch prices of rare disease therapies; this policy proposal would have a devastating impact on rare disease patients and limit access to transformational treatments.

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## Rare Disease Treatment Developers Face Significantly More R&D Challenges

From identifying patients, to enrolling patients in clinical trials, to ensuring patients have the support they need to access care and treatment, the challenges facing rare disease drug companies developing life-changing treatments are significantly greater than the tall order facing developers of therapies for more common diseases.

Economies of scale are unfavorable, yet research and development costs remain the same, if not higher, due to the lack of natural history, complex diagnosis and limited access to people living with that rare disease. As a Coalition, we collectively invest on average 58 percent [of operational expenses] into research and development and need to rely on third-party capital to continue to innovate. If price controls target rare disease companies, less money will be invested, and such investments would not be directed to rare disease research.

## Policymakers Should Pursue Approaches That Recognize the Need for Financial Sustainability and Improving Patient Outcomes

Rare disease drug development necessitates a different business model, driven by the need to balance financial sustainability and future innovation. As a result, arbitrarily capping launch prices will only undermine rare disease treatment development and further limit access for patients with significant unmet needs. These arbitrary price controls, alongside any additional regulations for rare disease drug development and approval, will disincentivize future investments necessary to develop new treatments for the majority of patients who currently lack options and negatively impact the promising innovation currently underway.

Policymakers should pursue approaches that appropriately balance the need to manage and predict drug spending without compromising improved patient access and outcomes, and encourage more innovation for the millions of rare disease patients with unmet needs.