# The Unintended Consequences of Drug Pricing Policies on Orphan Drug Development

Rare diseases impact **nearly 1 in 10 people in the US** and are largely untreated, with 95 percent lacking FDA-approved therapies. Orphan drug development is inherently challenged by scientific, clinical, and market barriers that impede the development of new rare disease therapies, including small patient populations, complex trial designs, and high research and development (R&D) costs. Policymakers have traditionally come together to help companies overcome these challenges and pass bipartisan policies such as the Orphan Drug Act of 1983 and subsequent legislation to support rare disease patients.

Recent government pricing policies undermine **40 years of bipartisan support** for rare disease patients

**40** research programs discontinued from 2022 to 2023, and biotech seed and series A funding that went to rare disease companies dropped to **2%**

R&D budgets for orphan drugs may **decline 18.5%** by 2039 and there may be a **projected 40% drop** in orphan drug approvals by 2035

Policies establishing a "Maximum Fair Price" or "Upper Payment Limit," like those found in the Inflation Reduction Act and state Prescription Drug Affordability Boards, negatively impact the rare disease community. Specifically, these policies:

- Shrink R&D budgets and investments for orphan drug development
- Disproportionately impact small companies that historically lead in orphan drug development
- Disincentivize the pursuit of orphan drug indications first in drug development
- Discourage the interest of subsequent orphan drug indications for approved treatments
- Shorten life expectancies and encourage an increase in off-label use
- Distort complex drug supply chains once an orphan drug is approved

Federal and state policymakers must recognize the disproportionate impact pricing policies have on rare disease patients. A return to bipartisan support is essential to sustain innovation and ensure equitable access to life-saving treatments for rare disease patients. Policymakers should consider the following policy alternatives to foster orphan drug development:

Maintain incentives for rare disease research

- Increase the Orphan Drug Tax Credit to its original 50 percent of qualified R&D costs
- Reauthorize the Rare Pediatric Disease Priority Review Voucher program
- Expand direct federal funding and involvement in rare disease product research and development

Carve out all orphan drugs from pricing policies

- Improve the Orphan Drug Exclusion to encompass all products designated solely to treat rare diseases
- Encourage state Prescription Drug Affordability Boards to exclude drugs solely focused on rare diseases from Upper Payment Limits

Develop policies to enhance orphan drug access

- Bolster outcomes-based contracting

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