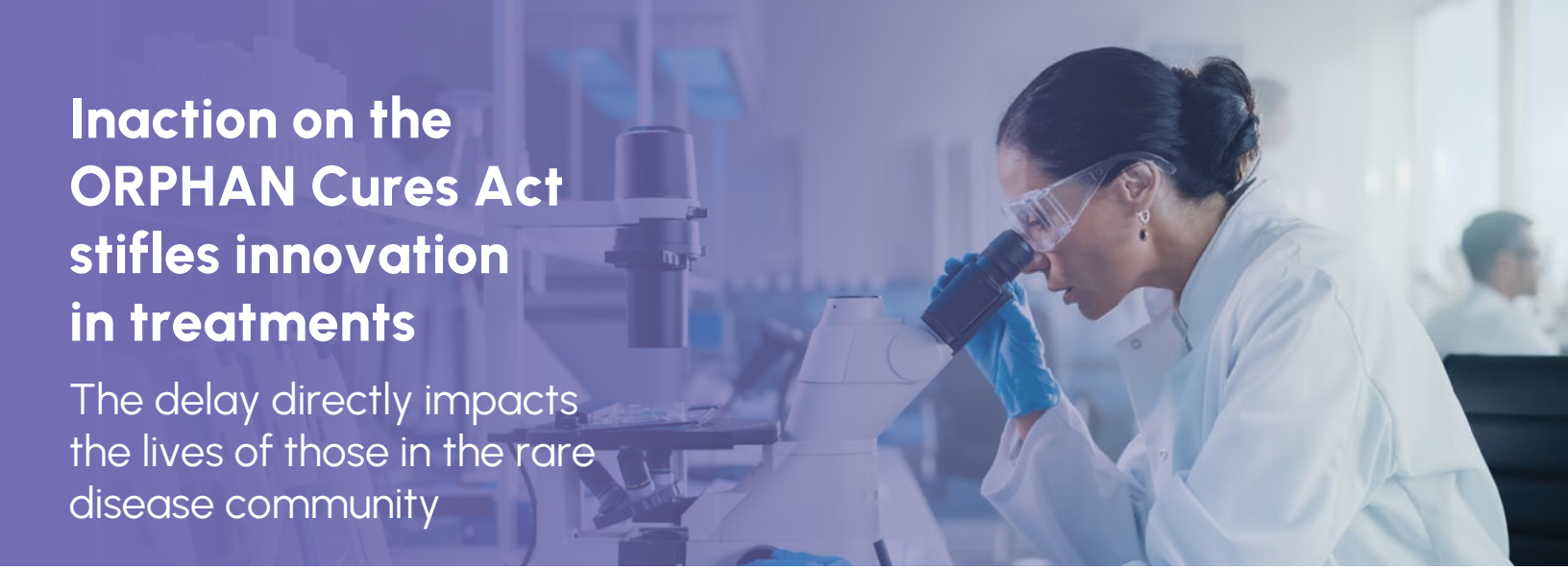

Inaction on the ORPHAN Cures Act stifles innovation in treatments

The delay directly impacts the lives of those in the rare disease community

New federal barriers undermine patient access to life-changing or life-saving medications

Under Medicare's Drug Price Negotiation Program, only orphan drugs that treat one rare disease or condition would be excluded from price negotiation.

Without a federal policy update, a single orphan medicine that could be used to treat more than one additional rare disease would be subject to price negotiation.

This discourages the development of rare disease and rare cancer therapies and diminishes access to life-changing treatments for patients who suffer from rare diseases.

The ORPHAN Cures Act (H.R. 946) would ensure that drugs used to treat one or more rare diseases will not be subject to government price setting under the Inflation Reduction Act and clarify the timeline used to determine when an orphan drug may become eligible for negotiation, further incentivizing the innovation of necessary rare disease treatment.

Significant investments already made

ORPHAN Cures protects the significant progress made for rare disease patients, ensuring that the legacy of access to therapies and the substantial investments in research are not put at risk.

Thousands of patients living with rare diseases

ORPHAN Cures facilitates new therapies for rare disease patients, ensuring these individuals and their families are not overlooked in favor of more common conditions.

Renewed hope and security

ORPHAN Cures brings hope to patients and their families by improving access to evidence-based, FDA-approved therapies without which they face debilitating disease progression and demanding caregiving responsibilities.

Support bipartisan, federal legislation to protect research into treatments for the 30 million Americans currently suffering from one of more than 10,000 rare diseases and rare cancers.

Save Rare Treatments

To learn more about the ORPHAN Cures Act or rare disease and rare cancer, please visit https://www.saveraretreatments.org

Patients with rare diseases must not be overlooked

When elected leaders focus on addressing rare disease policy, patients and their families benefit. **One in 10 Americans is affected by a rare disease**, which is a single disease that affects fewer than 200,000 people in the United States.[1]

Roughly 30 million Americans are living with a rare disease, yet less than 10% of rare diseases have an FDA-approved treatment option or an orphan drug.[2] An orphan drug is developed specifically to treat, prevent, or diagnose a rare disease or condition, often referred to as an "orphan disease." Thousands of rare disease patients are waiting for new treatments to come into existence to meet their specific medical condition. Patients also rely on researchers to identify new uses for existing medications that could treat their rare condition.

1 in 10
Americans is living with a rare disease

> 10,000
known rare diseases and rare cancers[3]

1 in 2
patients diagnosed with a rare disease is a child[4]

POLICY INCENTIVES FOSTERED SIGNIFICANT ADVANCEMENTS

With the right policy environment, rare disease advancements can flourish

In 1983, Congress passed legislation to foster development of drugs for rare diseases, resulting in significant investment and advancements in rare disease research:[5]

Patients benefited from access to life-saving therapies for rare diseases and rare cancers as companies were encouraged to make drugs for small patient populations with few, if any, options available.

The number of federally approved orphan drugs **increased by 1,576% – from 38 to more than 880 drugs** that treat over 1,200 rare disorders.[6]

Life-changing treatment

Research into additional uses for orphan drugs can take years but have immense impacts on patients. Yet, the Inflation Reduction Act discourages rare disease research and overlooks the rare disease community. Specifically, federal government price negotiations could:

Reduce overall medication research and development spending by as much as **$663 billion**.[7]

Lower annual spending on cancer research and development by **$18 billion per year or approximately 32%**.[8]

Lose 330 million collective years among rare disease patients by 2039.[9]

[1] "Orphan Drugs in the United States: An Examination of Patents and Orphan Drug Exclusivity." *National Organization for Rare Disorders*, NORD, 2021

[2] Wan, Eric L et al. "Zebras Among Us: Advocating for the 30 Million Americans Living with Rare Disease." *Medical science educator* vol. 33,5 1239-1242. 15 Aug. 2023, doi:10.1007/s40670-023-01856-2

[3] "List of Rare Diseases: A-Z Database: Nord." *National Organization for Rare Disorders*, NORD

[4] "Show Your Stripes® for Rare Disease Day with NORD." *National Organization for Rare Disorders*, NORD, Oct. 2024

[5] The Orphan Drug Act. Public Law 97-414

[6] "Recognizing the 40th Anniversary of the Orphan Drug Act, the Rare Disease Company Coalition Calls on Policymakers to Renew Commitment to Advancing Innovation for Rare Disease Patients." *Rare Disease Company Coalition*, 17 Apr. 2023

[7] Zinberg, Joel. "The Arrival of Medicare Drug Price Controls: No Cause for Celebration." *Paragon Health Institute*, 6 Sept. 2023

[8] Philipson, Tomas, and Troy Durie. "Issue Brief: The Impact of HR 5376 on Biopharmaceutical Innovation and Patient Health." University of Chicago, 2021

[9] Philipson, Tomas, Yier Ling, and Ruiquan Chang. "The Impact of Recent White House Proposals on Cancer Research." University of Chicago, 2022

Save Rare Treatments

To learn more about the ORPHAN Cures Act or rare disease and rare cancer, please visit https://www.saveraretreatments.org