



The prices for orphan drugs used for more than one disease are currently eligible for price negotiation – which discourages research and development for life-saving innovations for rare patients.

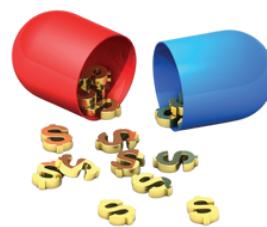
The ORPHAN Cures Act would preserve financial incentives for companies that invest resources into orphan drugs through two strategies:

1. Exclude orphan drugs from price negotiation (Orphan Drug Exclusion) as long as their FDA-approved uses are **exclusively for rare diseases**
2. Specify that the **countdown for price negotiation eligibility starts at the date of a product's first non-rare approval**

These narrowly targeted changes will ensure that investment in rare disease research and the pursuit of new uses for existing drugs can still lead to both sustainable profits for companies and treatments and cures for patients.

Across the rare disease community, individuals living with rare disease and their families are concerned about the impact that potential price negotiation is already having on investments and pipeline decisions for rare disease treatments.

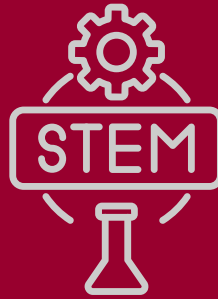
By one estimate, price negotiation would lead to a *reduction* in research and development spending by \$663 billion, resulting in 135 fewer new drugs being approved through 2039. The ORPHAN Cures Act is critical to reverse this trend.



The ORPHAN Cures Act

The ORPHAN Cures Act is **bipartisan legislation** that would maintain existing incentives to boost research into new treatments for rare diseases (“orphan drugs”). 30 million Americans currently suffer from one of more than 7,000 rare diseases. Because of the small populations affected by rare conditions, it would not be profitable to develop drugs for these conditions through the standard system – which would leave many patients without the promise of treatment options.

How the ORPHAN Cures Act Saves the American Economy and Lives



GROWING THE AMERICAN WORKFORCE

Job Creation

The pharmaceutical manufacturing industry directly *employs* approximately 291,000 workers in the United States.

Employment Multiplier Effect

Each job in pharmaceutical manufacturing supports an additional 4.1 jobs in the broader economy. Including these indirect and induced jobs, *the industry supports one in every 20 jobs in the U.S. economy.*

MAINTAINING AMERICAN LEADERSHIP IN INNOVATION

Research & Development Investment

In 2021, pharmaceutical companies invested more than \$102 billion in R&D, accounting for 17.1% of total R&D investment in the United States.

The industry invests 16.6% of its sales back into R&D, nearly 3.5 times more than the average U.S. industry.

STEM Employment

Approximately 25% of all jobs in pharmaceutical and medicine manufacturing are STEM-related, compared to 6.6% in the overall U.S. workforce.

STRENGTHENING THE AMERICAN ECONOMY

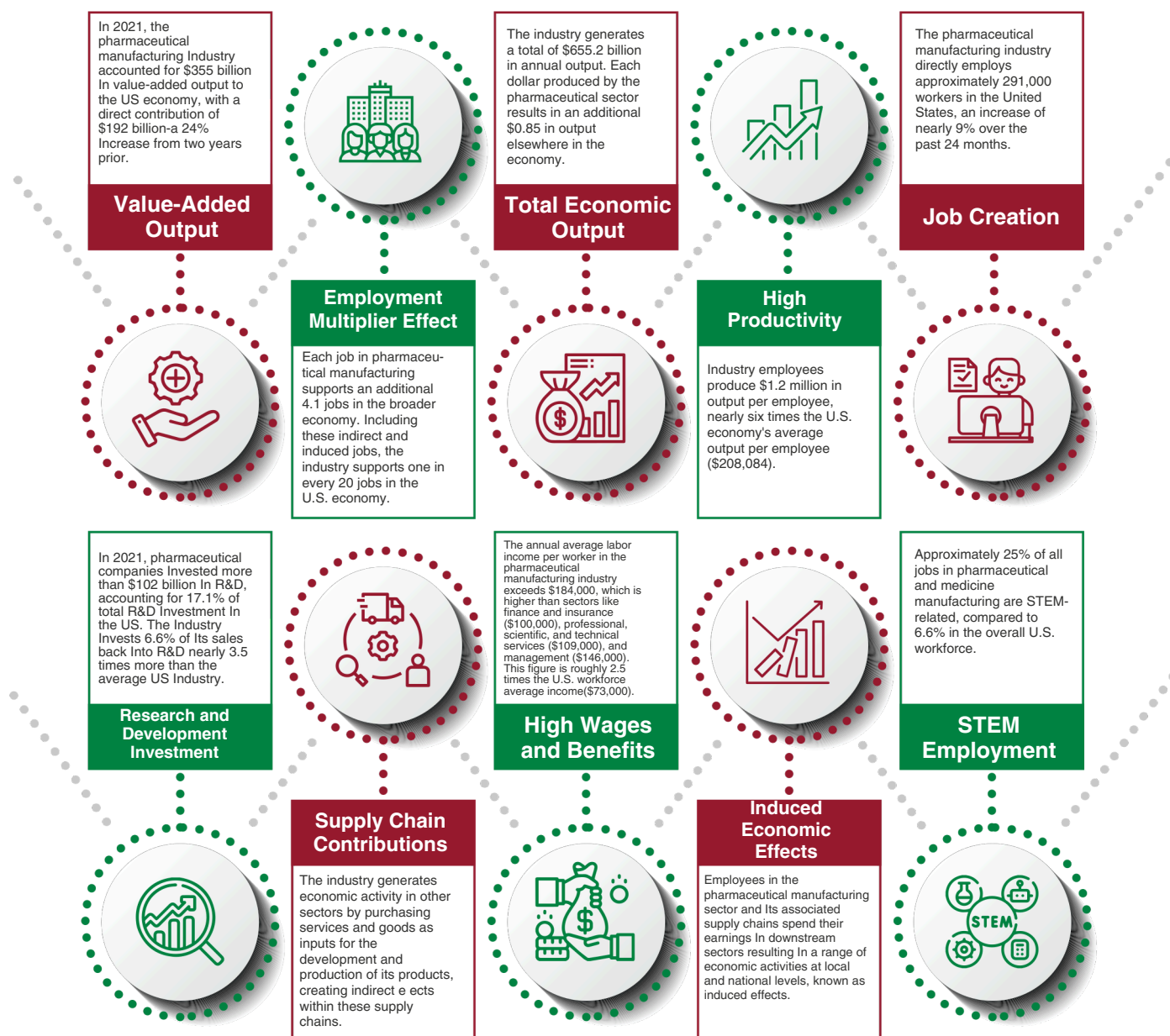
Value-Added Output

In 2021, the pharmaceutical manufacturing industry accounted for \$355 billion in value-added output to the U.S. economy, with a direct contribution of \$192 billion—a 24% increase from two years prior.

Total Economic Output

The industry generates a total of \$655.2 billion in annual output. Each dollar produced by the pharmaceutical sector results in an additional \$0.85 in output elsewhere in the economy.

American Economy: The ORPHAN CURES Act Creates and Saves American Jobs!



Philipson, T., Ling, Y., & Chang, R. (2022). "The Impact of Recent White House Proposals on Cancer Research." University of Chicago.

The American Economy and Saving Lives: Impact of IRA on Drug Development

1. Revenue Projections: *The IRA is projected to reduce the lifetime revenue of small molecule drugs by 5% to 6% and biologics by 3% to 4%, potentially leading to decreased investment in rare disease drug development.*

2. Incentives for Orphan Drugs: *The IRA exempts orphan drugs approved for a single indication from price negotiations. However, this exemption applies only to drugs with a single approved indication, which may discourage manufacturers from pursuing additional rare indications!*