

Maintaining Progress for Californians with Rare Disease: The ORPHAN Cures Act

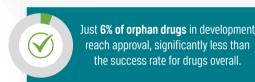
Congress should prioritize fixing the IRA's orphan exemption by passing the Optimizing Research Progress Hope and New (ORPHAN) Cures Act (H.R.5539).

An Exemption Needs to Account for the Reality of Orphan Drug Development

In recognition of the need to protect orphan drug development, the *Inflation Reduction Act (IRA)* provides a specific exemption for government price setting for medicines with a **single** orphan designation and indications only with that designation. The reality is, that exemption is far too narrow to meet the needs of the more than 3 million rare disease patients waiting for treatments in California.

Orphan Drug Research and Development (R&D)

R&D for small treatment populations is highly risky and complex, resulting in failure more often than other kinds of conditions. Because of these challenges it takes **nearly 4 years** longer to develop an orphan drug compared with medicines to treat more common conditions. In the absence of sufficient incentives to encourage their development, these medicines would not exist.



CHALLENGES IN ORPHAN DRUG DEVELOPMENT



Small Patient Populations



Complex Scientific Challenges of the Disease



Difficulties diagnosing patients and recruiting for clinical trials

The *Orphan Drug Act* (ODA) Incentivizes Post-Approval R&D

The bipartisan passage of the ODA in 1984 encouraged development of orphan drugs because existing market incentives are often insufficient to attract the necessary investments.

The current exemption does not protect orphan drugs developed as a result of post-approval R&D. Many treatment options for rare disease patients are available because of post-approval R&D.

Prioritizing Unmet Need for Patients

While significant progress has been made, fewer than 10% of rare diseases have available treatment options, underscoring the importance for orphan drug incentives.



Since the enactment of the ODA **more than 600 orphan drugs** have been
approved in the U.S. compared with just
10 in the decade before passage.



Over a third of orphan drugs had multiple FDA approved indications; half of all subsequent approvals for orphan drugs came 5 years after initial approval.