CALIFORNIA LIFE SCIENCES

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.

Just **6% of orphan drugs** in development reach approval, significantly less than the success rate for drugs overall.

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.

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.

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.

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.