# PRESERVING THE ORPHAN CURES ACT

## MAINTAINING PROGRESS MADE FOR CALIFORNIANS WITH RARE DISEASE

CALIFORNIA LIFE SCIENCES

**Congress should preserve strong incentives for rare disease innovation by protecting the orphan drug exemption from Medicare price setting and opposing policies that undermine continued research and development, such as H.R.6166, the *Lowering Drug Costs for American Families Act*, and S.3019, the *No Big Blockbuster Bailouts Act***

### MYTH

The original single indication exemption in the *Inflation Reduction Act* (IRA) for orphan drugs was sufficient to maintain incentives for efficient, science-based development of orphan drugs.

### FACT

The IRA's Medicare Drug Price Negotiation Program (MDPNP) initially limited the orphan drug exemption to single-indication therapies, discouraging development of additional rare disease uses. The enactment of the *ORPHAN Cures Act* in 2025 corrected this by extending the exemption to subsequent rare disease indications, preserving incentives for continued R&D. **Two recently introduced bills, S.3019 and H.R.6166, would repeal the *ORPHAN Cures Act*, reversing this fix and weakening future orphan drug development.**

### UNIQUE CHALLENGES



Just **6% of orphan drugs** make it to an approval, significantly less than the success rate for drugs overall.

### DESIGNATION ≠ APPROVAL



**Orphan drug development is iterative.** A designation qualifies sponsors for critical incentives but does not guarantee an approval.

### MYTH

Under the *ORPHAN Cures Act,* all orphan drugs are exempt from the MDPNP.

### FACT

The *ORPHAN Cures Act* fixed the exemption so that an orphan drug only becomes eligible for the MDPNP once the drug **receives a non-orphan indication** approved by the FDA. It does not allow for medicines with broad indications to remain exempt. **This maintains the critical incentives that were intended for the development of these exceptionally challenging medicines.**

### CRITICAL RESEARCH INCENTIVES



This bipartisan law is already driving renewed R&D investment, with investor interest reversing from a low 56%, climbing back up to **73% reporting renewed interest in rare disease therapies.**

### CONTINUAL PROGRESS



**Over a third** of orphan drugs had **multiple FDA approved indications**; many of which came 5 years after initial approval.

### A MISGUIDED CUT




More than **$2.1 billion has been invested** in rare disease innovation since enactment of the *ORPHAN Cures Act,* and repealing it would jeopardize this progress.