THE ORPHAN CURES ACT
MAINTAINING PROGRESS FOR CALIFORNIANS WITH RARE DISEASE

CALIFORNIA LIFE SCIENCES

Congress should prioritize fixing the IRA's orphan exemption by passing the ***Optimizing Research Progress Hope and New (ORPHAN) Cures Act (H.R.946).***

MYTH

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

FACT

Current law exempts orphan drugs with a single orphan designation and single approved indication for that designation from being eligible for the Medicare Drug Price Negotiation Program (MDPNP). When an orphan drug receives a second orphan designation by the FDA, the drug is no longer exempt from the MDPNP. **However, this contradicts the iterative scientific nature of developing medicines for small patient populations.**

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 will be exempt from the MDPNP.

FACT

The *ORPHAN Cures Act* would fix 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 would 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.**

MYTH

When an orphan drug becomes eligible for the MDPNP, the clock until the drug is eligible for selection (either 7 or 11 years) starts at the time the drug loses the exemption.

FACT

Under the current law, once an orphan drug becomes eligible for negotiation, the countdown clock until it can be selected begins with the date of the drug's initial approval. Starting the clock at the date of the initial approval, instead of when the exemption is lost, is premature and disincentivizes additional development after its initial approval. **The *ORPHAN Cures Act* fixes this so that the clock appropriately begins when the orphan drug loses its exemption.**

CONTINUAL PROGRESS

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