# Minor changes to the Inflation Reduction Act (IRA) will make a big difference for rare disease patients

SUPPORTING PATIENT ADVOCACY ORGANIZATIONS:

EveryLife Foundation for Rare Diseases

NORD® National Organization for Rare Disorders

For the first time, empowered by the Inflation Reduction Act of 2022 (IRA), the Centers for Medicare & Medicaid Services (CMS) will negotiate the price of some prescription drugs. The IRA included a limited exclusion for some rare disease therapies from the negotiation program, but the narrow nature of that exclusion could undermine some of the critical incentives established by the Orphan Drug Act.

Under current law, orphan drugs that have only one orphan designation AND one approved indication (or multiple approved indications all tied to the same designated rare disease) are excluded. However, as soon as the drug is designated for a second disease, even without any associated approvals, it will become negotiation eligible—and the clock for when a product could be selected for negotiation starts at the date of the drug's very first approval, even if the second designation or additional approval occurs many years later.

*Two technical changes to the IRA will help preserve the hope of the 95% of rare disease communities without disease-specific FDA approved treatment options, yet will not change the number of approved indications a product can have before becoming eligible for Medicare negotiation.*

## CONGRESSIONAL ASK #1:

**Clarify that the number of orphan designations FDA granted to a product has no effect on its eligibility for the IRA's orphan drug exclusion.**

- Designations open access to incentives to conduct further clinical research to see if a product could be an effective treatment in additional populations or diseases.
- Designations do NOT allow a product to be marketed and therefore should not be tied to any policies affecting price or access of approved products.

## CONGRESSIONAL ASK #2:

**Maintain the purpose of the orphan drug exclusion by clarifying an orphan product becomes negotiation-eligible 7 or 11 years after it loses that exclusion.**

- Tying eligibility for negotiation strictly to an orphan product's first approved indication eliminates any incentive for drug companies to pursue additional rare disease indications.
- Ensuring the clock doesn't start until the exclusion is lost may encourage drug companies to continue to study a drug's potential to treat additional rare diseases.

## DESIGNATIONS AND APPROVED INDICATIONS ARE NOT THE SAME

| | ORPHAN DRUG DESIGNATION | FDA-APPROVED INDICATION |
|---|---|---|
| **TIMING** | **Early** in drug development | At the **end** of drug development and FDA premarket review |
| **PURPOSE** | **Unlock R&D incentives** such as:<br>• Tax credits for qualified clinical trials<br>• Exclusion from FDA user fees<br>• Potential seven years of market exclusivity **if approved** | Allow for **marketing** of the drug in the US |
| **REQUIRED EVIDENCE** | The target disease affects **fewer than 200,000** Americans & there is some preclinical or early clinical evidence that the drug candidate may plausibly work for the rare disease | Through extensive clinical studies in patients with the target disease, the drug has been **shown safe and effective** for its intended purpose |
| **NEXT STEPS IN THE PRODUCT'S LIFE CYCLE** | **Study** the drug in patients with the target disease | Begin to **market** the drug in the US |
| **EXAMPLE** | *Treatment of rare disease x* | *Treatment of rare disease x in patients 12 years and older who have confirmed mutation AB* |
| **SINCE PASSAGE OF THE ODA IN 1983** | **6500+ orphan designations** | **1150+ approved indications** |

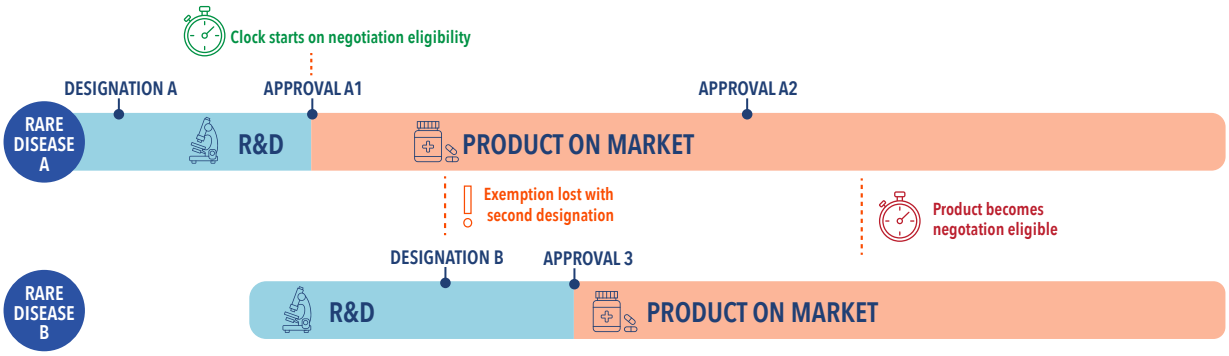
# The IRA's narrow orphan drug exclusion threatens continued rare disease drug development and repurposing efforts

Example: One small molecule orphan drug candidate with R&D programs for two different rare diseases

## WHAT DOES AN EXEMPT DRUG LOOK LIKE UNDER CURRENT LAW?

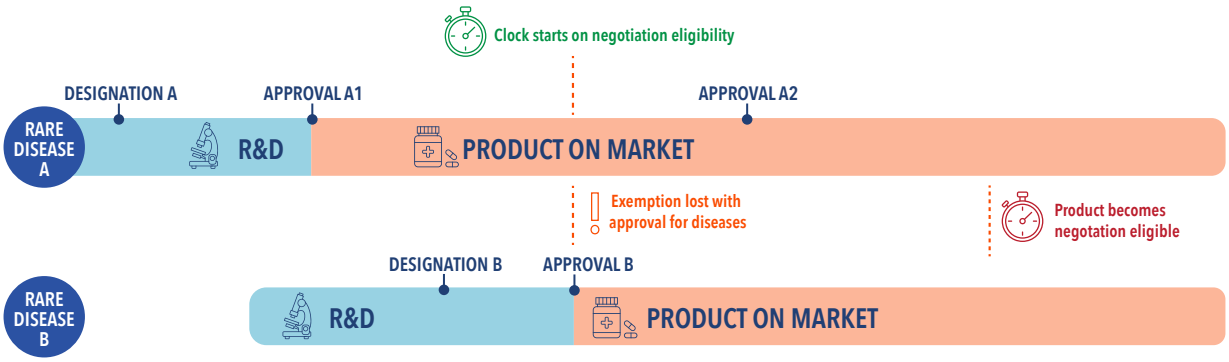

Drug exempt from negotiation

## WHEN DOES A DRUG LOSE EXEMPTION UNDER CURRENT LAW?




- Drug loses exemption when a second designation is granted in 2012.
- It becomes negotiation eligible in 2018 – seven years after its first approval.
- If chosen, negotiated prices start in 2020 – nine years after its first approval.

## WHEN DOES A DRUG LOSE EXEMPTION UNDER PROPOSED LAW?




- Drug loses exemption when a second APPROVAL is granted in 2014.
- It becomes negotiation eligible in 2021 – seven years after its SECOND approval.
- If chosen, negotiated prices start in 2023 – nine years after its SECOND approval.


For too many Americans living with rare diseases, out-of-pocket prescription drug costs create significant financial barriers to access. However, approximately 95% of all known rare diseases lack an FDA approved treatment. **The hope of millions of Americans with unmet medical needs rests in continued research and development into new and better therapies**. Recognizing this dire need and the unique challenges that complicate rare disease drug development, a succession of Administrations and Congresses over the last 40 years have created and maintained incentives and policies to encourage more R&D into rare disease treatments.

That is why the IRA included a very narrow exclusion for some rare disease therapies from the Medicare Drug Price Negotiation Program (MDPNP). Unfortunately, that exclusion did not fully protect the orphan product incentives we've worked so hard to establish and maintain. The good news is that minor changes will make a big difference for rare disease patients.

**Some aspects of the IRA will help make it more affordable for rare disease patients with Medicare to access the therapies they need. Our organizations strongly support provisions such as capping annual out-of-pocket costs and making it possible to spread out monthly costs for Medicare Part D starting in 2025.**


For more information please contact:
Karin Hoelzer – khoelzer@rarediseases.org // Jamie Sullivan – jsullivan@everylifefoundation.org