RARE READY

A GENETIC CONDITION COALITION

State efforts to create Prescription Drug Affordability Boards (PDABs), while intended to make drugs more affordable, limit access to critical medical innovations. The implications are most profound for those living with a rare disease.

The Challenge

- **95%** of rare and genetic disorders lack FDA-approved treatments.
- Patients with rare conditions often have **limited or no alternatives** for care.

Impact of PDABs

- PDABs **do not** lower patient copayments, reduce premiums, nor do they create health system transparency.
- PDABs limit patient access to life-changing or life-saving medications. Patients with rare conditions often depend on these therapies as their **only treatment option.**
- PDABs threaten the development of future rare disease treatments by creating pricing uncertainty for medications that serve **small patient populations.** Products for these vulnerable patient populations, such as plasma-derived therapies, require significant investment and depend on complex manufacturing.
- PDABs strip away the patient protections afforded by orphan drug designation, leaving individuals with rare conditions at greater risk of **losing access to the only therapies designed to treat them.**

Impact of Upper Payment Limits

- Patients may face delays or denials in accessing necessary medications as cost-driven restrictions increase.
- Patients with rare diseases, who already have limited treatment options, will suffer from restricted drug availability.

Why This Matters

- Patients with rare diseases face disproportionate risks when innovation stalls.
- State legislators must consider how policies affect vulnerable populations.

Our Recommendations

- Gather advice from the rare disease community before passing PDAB legislation.
- Ensure PDAB legislation includes protections for rare disease patients to safeguard continued innovation and critical access to therapies.
- Ensure all rare disease therapies are exempt from PDABs.