Encouraging Innovation

for Rare Disease Patients

Only 5% of rare diseases have an FDA-approved treatment option, making additional innovation crucial.





Patients with rare diseases rely on policies that encourage research and development of treatment options.

Policymakers can encourage innovation through:



Preservation of User Fee
Acts that expand the
capacity of the FDA to
approve drugs



Support for policies that limit unintended consequences of the Inflation Reduction Act



Protection of the Accelerated Approval Pathway which speeds up FDA review

The future of rare disease relies on patient-centered policies.



