



DRUG PRICING PRINCIPLES

1. **The policy must maintain FDA's standards of safe and effective therapies.** Rare disease patients in the United States rely on the quality and safety of FDA-approved medicines. Any policy to lower the cost of therapies must not lower the safety and effectiveness of the medicines rare disease patients need.
2. **The policy must not raise out-of-pocket costs for rare disease patients.** Policies to lower the cost of prescription drugs must not inadvertently raise patients' out-of-pocket costs.
3. **The policy must not decrease patients' coverage of necessary therapies prescribed by their provider.** Some policies may negatively impact insurance coverage for rare disease treatments, making these treatments unaffordable and, therefore, inaccessible for patients with rare diseases. Patients should be able to access the therapy that they and their physician believe is right for them.
4. **The policy must not place an undue burden on innovation of new therapies.** Rare disease patients rely upon and, in many cases, their lives depend on continued innovation by the pharmaceutical industry. However, innovation is meaningless to patients if they cannot afford the therapies that are ultimately developed. Efforts to improve affordability and lower costs should be balanced with the need to sustain innovation.
5. **The policy must be supported by reliable data.** Policy interventions must be well-researched and supported by empirical evidence.