Rare Disease
Company Coalition

Accelerated Approval & Rare Diseases

Established in 1992 and codified by Congress in 1997, the U.S. FDA's accelerated approval pathway allows drugs for serious conditions that address an unmet medical need to be approved based on a surrogate endpoint — a measure that is reasonably likely to predict a clinical benefit, which is then confirmed in postmarketing studies. In 2012, Congress recognized the significant challenges of developing treatments for rare diseases and reinforced the availability of this pathway for bringing treatment options to rare disease patients.

FDA's accelerated approval pathway understands that a "one-size-fits-all" traditional model cannot always work - especially for serious and unique rare diseases that impact very small population sizes and progress slowly and variably from patient to patient. The accelerated approval pathway provides a science-based approach to overcome these challenges and meet the needs of patients while upholding FDA's rigorous standards for safety and efficacy.

The Promise for Rare Diseases

- Although biopharmaceutical researchers have made tremendous progress, 93 percent of rare diseases still do not have any treatment options, representing a significant unmet need for patients.
- Rare diseases present specific challenges - such as small population sizes, or slow, irreversible, and variable disease progression - that make it difficult to study in some cases using clinical measures as endpoints.
- Accelerated approval is a critical tool, well-suited to recognize these unique circumstances and, much as the pathway has changed the trajectory of disease for many cancer and HIV/AIDS patients, it holds enormous promise to transform the course of rare disease where current treatments don't yet exist.

Preserve and Uphold a Critical Pathway for Patients

Upholding the integrity of the accelerated approval pathway requires renewed focus on keeping patient access front and center, bolstering FDA's existing governance and science-driven implementation of this pathway, and continued attention to overcoming hurdles for sustained innovation. Any new policy proposals should:

- Acknowledge and address any long-term impediments to access to treatments for rare disease patients. Failure to recognize that accelerated approval therapies meet the full statutory standards for safety and effectiveness could lead to access challenges for patients.
- Account for evolving science and dynamic data generation. Scientific progress and increased disease understanding has led to the growing relevance and reliability of biomarkers in assessing effectiveness in certain circumstances. Real-world data can also be an important tool in helping to confirm the surrogate's predicted clinical benefits.
- When appropriate, continue to incentivize use of this pathway for certain rare diseases. Smaller biotech companies are leading innovators yet accelerated approval is already not attainable for many rare disease therapies because of the lack of agreed-upon surrogate endpoints. Assessing higher rebates on accelerated approval drugs would only further disincentivize development of some rare disease treatments and make the delivery of treatments to patients through this pathway even more difficult.