Rare Disease
Company Coalition

Support Accelerated Approval to Provide Timely Access to Treatment for Rare Disease Patients

The FDA's accelerated approval pathway recognizes that a "one-size-fits-all" traditional model for the approval of new medicines cannot always work - especially for rare diseases. This well-established, proven, scientifically rigorous path forward enables rare disease companies to make promising new and improved treatments available to patients at the earliest possible point in time for use in life-threatening and serious rare diseases.

Established in 1992, 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, which can be used to predict a clinical benefit in how patients feel and function, which is then confirmed in postmarketing studies. Surrogate endpoints are often used when a clinical benefit would take too long to measure and provide a science-based indicator that can be used to bring medicines to patients faster. This is especially important for diseases with no or limited treatment options, where time is of the essence.

In 2012, Congress recognized the significant challenges of developing treatments for rare diseases and reinforced the availability of this pathway for rare diseases. Rare diseases present specific challenges that make it difficult to study in some cases using clinical measures as endpoints, such as small population sizes, which makes it difficult to enroll large clinical studies, or slow and variable disease progression. Congress, the FDA, and the scientific community have all recognized the important role of surrogate endpoints as reliable biomarkers to assess effectiveness in circumstances where verification of clinical benefit may take many years as is the case with slowly progressing, debilitating rare diseases.

The accelerated approval pathway enables rare disease companies to make promising new treatments available to patients at the earliest possible point in time for use in life-threatening and serious rare diseases. In recent years, private and public payers have sought to restrict access to drugs approved through the accelerated approval pathway and if patient access is restricted, manufacturers will have little incentive to pursue research and development in rare diseases, as an already small (rare) population becomes smaller. Researchers recently found that substantial changes to the accelerated approval pathway, proposed by state Medicaid programs, congressional advisory bodies and other stakeholders, will render the potential development of therapies for most untreated orphan conditions economically untenable.

The Promise for Rare Diseases

- Although biopharmaceutical researchers have made tremendous progress, **more than 90 percent of rare diseases still do not have any treatment options,** representing a significant unmet need for patients.
- Rare diseases present specific challenges that, in some cases, make it difficult to conduct trials using clinical measures as endpoints. This underscores the utility of using surrogate endpoints that can measure likely clinical benefit.
- While **less than 10% of all accelerated approvals in its 30-year history have been used for non-oncology rare diseases**, today's increased understanding of rare disease and advances in targeted drug development are making it possible to realize the promise of accelerated approval for rare diseases where current treatments don't yet exist.

We Support Policy Proposals That Reinforce the Accelerated Approval Pathway, Enhance Regulatory Reliability, Provide Access to Patients, and Sustain Further Investment

As policymakers consider policy proposals that impact the trajectory of rare disease treatment development, we advocate for an approach that considers the unique circumstances of rare diseases and caution against punitive measures that could chill future investment and development of rare disease treatments.

- Recent analysis concluded that if FDA's accelerated approval pathway was not available or significant restrictions were placed on accelerated approval drugs it would likely result in as many as two-thirds of currently approved accelerated approval drugs failing to reach up to 3.6 million patients.[1]
- Recent proposals to restrict access to accelerated approval therapies within Medicaid could see up to 43% of investigational therapies no longer being developed, impacting up to 319,000 patients annually, who likely have no other treatment options.[2]

Delaying access due to the use of surrogate endpoints in accelerated approval will have a devastating impact on patient access and will provide limited cost savings for states.

- The percentage spent on treatments approved under accelerated approval is **less than ½ of 1% of US states' overall Medicaid budget.**[3]
- The value of bringing a treatment to market provides significantly more cost savings for society given the burden range of rare diseases is estimated to be between **$7.2 trillion to $8.6 trillion per year**[4] in the United States.

WE SUPPORT:	WE OPPOSE:
Early engagement, agreement, and initiation of confirmatory studies prior to approval when feasible	Arbitrary deadlines for completion of confirmatory studies and withdrawal procedures that are automatic or otherwise lack due process and patient input
Using Real World Evidence and surrogate endpoints to inform verification of clinical benefits	Product labeling requirements that could confuse clinicians and patients, burden the FDA, and lead to restrictive payer coverage policies
Enhanced visibility of confirmatory trial progress leveraging existing mechanisms (e.g. annual report)	Reporting requirements that increase administrative burden and fail to account for the slow and variable progression of some rare diseases
Regulatory process improvements to support consistent and optimal use of the pathway to facilitate the availability of treatments for rare diseases	Policies that limit patient access to accelerated approval drugs and undermine FDA's statutorily defined decision making authority as arbiter of safety and effectiveness
	Undercutting reimbursement, coverage and patient access to therapies developed under the accelerated approval pathway

[1] https://vitaltransformation.com/2022/06/calculating-the-value-and-impact-of-accelerated-approvals/
[2] https://vitaltransformation.com/2022/06/calculating-the-value-and-impact-of-accelerated-approvals/
[3] https://vitaltransformation.com/2022/06/calculating-the-value-and-impact-of-accelerated-approvals/
[4] https://www.rarecoalition.com/2022/03/15/the-case-for-rare-diseases-as-a-public-health-priority/

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