Rare Disease Company Coalition

Reinforce the Accelerated Approval Pathway for Rare Disease Patients

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. Accelerated approval can be credited with transforming the trajectory of deadly diseases such as HIV and many cancers while upholding the Agency's high standards for safety and effectiveness.

The pathway has proven successful in enabling earlier patient access to novel treatments, the vast majority of which have converted to traditional approval upon confirmation of clinical benefits (78% of accelerated approvals prior to 2020).[i]

FDA's accelerated approval pathway recognizes that a "one-size-fits-all" traditional model cannot always work - especially for serious and unique rare diseases with very small population sizes that progress slowly and variably from patient to patient. Therapies approved through the accelerated approval pathway are subject to the same stringent, evidence-based clinical review and approval standards as the traditional FDA approval process, as well as to post-approval confirmatory trials to verify clinical benefit.

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.[ii]
- Rare diseases present specific challenges that, in some cases, make it difficult to conduct trials using clinical measures as endpoints.
- 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.[iii]

We Support Policy Proposals That Recognize the Potential of Rare Disease Therapies and Reinforce the Role of the Accelerated Approval Pathway

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.

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 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

[i] GK Ragu, PhD. Presentation at the Rare Disease Legislative Advocates Congressional Caucus Briefing: The Accelerated Approval Pathway: Reflecting the Rare Disease Community's Priorities of Rigor, Safety, and Urgency. February 23, 2022. Available at https://www.youtube.com/watch?v=xiGgC8QHqSA

[ii] National Organization for Rare Disorders (NORD). BARRIERS TO RARE DISEASE DIAGNOSIS, CARE AND TREATMENT IN THE US: A 30-Year Comparative Analysis. November 19, 2020. https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf

[iii] EveryLife Foundation. Accelerated Approval Works One-pager. February 2022. https://everylifefoundation.org/wp-content/uploads/2022/04/Accelerated-Approval-Works-One-Pager.pdf