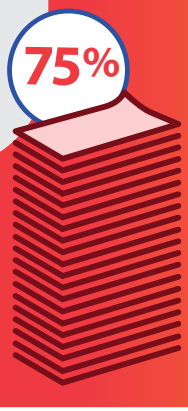
ACCELERATED APPROVAL IS... safe, effective, necessary, and working to deliver better health to those in dire need.

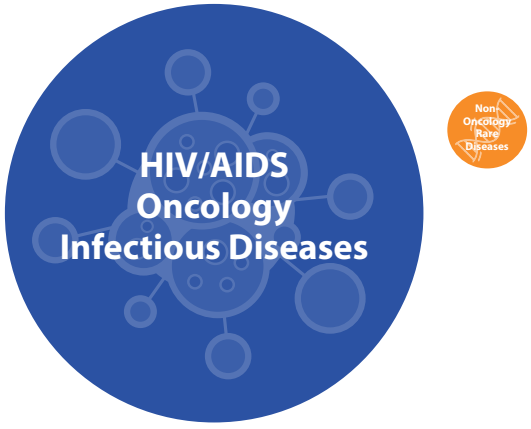
- In diseases where there was once "nothing to do" for patients, we now have opportunities to slow, halt or reverse disease progression, collect data and evaluate therapeutic impact over time, improve patient outcomes, and encourage scientific advances.
- The accelerated approval pathway is working as intended: delivering better health to those in dire need. We must value and protect it for the benefit of patients where the opportunities of accelerated approval have yet to be realized.
- NOW is the time to realize the promise of accelerated approval to advance therapies for patients with rare diseases and Congress should encourage reforms that unleash this potential, not stymie it.

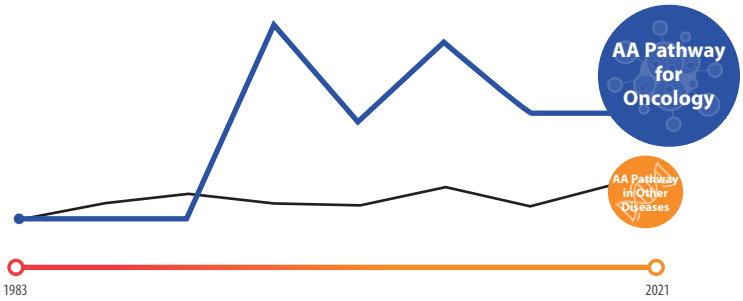
> "Accelerated Approval cannot just exist as a pathway. It must exist as a true possibility."
>
> *–Emily Milligan, Executive Director, BARTH Syndrome Foundation, 2021 Scientific Workshop*



More than 75% of all products approved via the AA pathway **have been converted to traditional approvals**, with many more still in the process of collecting data to support a full approval.

Less than 10% of all products approved via the AA pathway are indicated for non-oncology rare diseases





Oncology Diseases Use AA Pathway at a 3-5 Times Higher Rate Than All Others

Policies that regard treatments approved via the AA pathway as experimental **exacerbate health inequity** among communities eligible for potentially life-altering and lifesaving medications.

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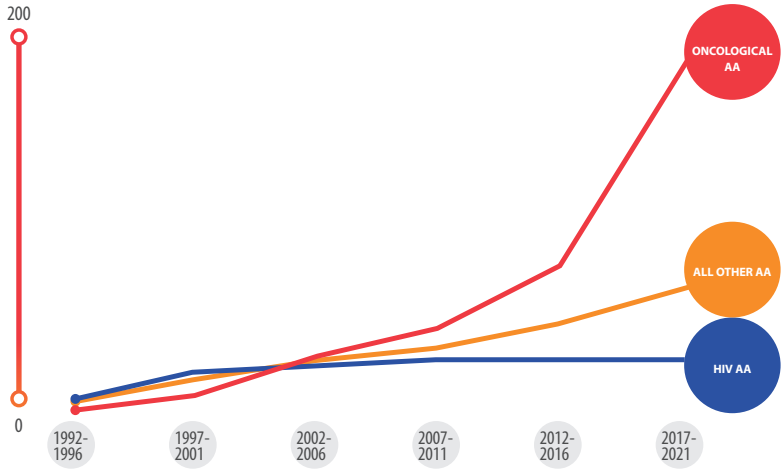
Early accelerated approval successes can catalyze more innovation in a disease area with high unmet needs as was experienced by HIV and oncology therapeutic development.

Accelerated Approval Trends



Accelerated Approval Works

29% drop
in cancer death rates from 1992-2017*

= **2.9 million fewer cancer deaths!**

*https://acsjournals.onlinelibrary.wiley.com/doi/10.3322/caac.21590

Accelerated approval may be the ONLY path forward for many rare diseases.

- Therapy development using biomarkers is not a compromise; surrogate endpoints are validated measures that are backed by science.
- Surrogate endpoints (biomarkers) can have advantages over the use of clinical outcomes. In many diseases, surrogate endpoints (biomarkers) can more accurately capture real-time disease progression or improvement.
- Well established biomarkers have enabled the accelerated approval pathway to transform oncology therapeutic development. Policy should support the prioritization of resources and collaboration necessary to advance knowledge on rare disease biomarkers and intermediate clinical outcomes
- The safety of rare disease treatments approved via the accelerated approval pathway is evaluated in the context of the established benefit risk framework for the target patient population. When weighed against the known natural disease progression and certainty of doing nothing, trial outcomes are carefully considered.
- Reducing access to treatments approved via the accelerated approval pathway would NOT address the biggest drivers of healthcare costs but WOULD harm patients with life-threatening conditions.

Accelerated approval is NOT a lower standard. Treatments approved via the AA pathway are **subject to the same statutory standards** for proving safety and efficacy as traditional drug approvals.

> Accelerated Approval can optimize the translation of science into rare disease medicines in a timelier fashion than the traditional FDA approval process allows."
>
> *–Dr. Emil Kakkis, Founder, EveryLife Foundation, 2021 Scientific Workshop*

Spending on drugs approved through the accelerated approval pathway accounted for **less than one percent** of annual Medicaid spending between 2007 and 2018.

Spending on accelerated approval drugs **remained steady at 0.6% to 0.8%** beginning one year after the 2012 passage of the Food and Drug Safety and Innovation Act, which expanded use of the pathway to rare diseases.**

**https://doi.org/10.37765/ajmc.2021.88596

EVERYLIFE FOUNDATION FOR RARE DISEASES

About the EveryLife Foundation

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.

everylifefoundation.org