# FDA'S EXPEDITED PROGRAMS:

## Getting Essential New Treatments to Patients

**Over the past 30 years, Congress has established five programs aimed at expediting patient access to therapies that treat serious or life-threatening conditions, while simultaneously upholding FDA's gold standard for safety and efficacy. Each of FDA's expedited programs has unique features and treatments may qualify for more than one.**

## The Elements of FDA's Expedited Programs

**Fast Track designation** (1988) helps facilitate the development and expedite the review of drugs that treat serious conditions and fill an unmet medical need. Allows early and frequent communication between the innovator and the FDA to help resolve questions and issues quickly.

**Priority Review designation** (1992) sets FDA's goal to act on a new drug application within six months (compared with 10 months under standard review) for drugs for serious conditions that would be significant improvements in the safety or effectiveness.

**Accelerated Approval pathway** (1992) allows drugs for serious conditions that address an unmet medical need to be approved earlier based on a surrogate endpoint — a measure that reasonably predicts how well a drug works – with a post-marketing confirmatory study required to verify the predicted clinical benefit.

> **"No matter what the approval pathway is, we consistently have the same statutory standards of ensuring safety and efficacy before a product is marketable in the U.S. Increased flexibility does not mean we're abandoning standards or quality."**
>
> **Former FDA Commissioner Margaret Hamburg**

**Breakthrough Therapy designation** (2012) expedites the development and review of a drug for a serious condition if evidence from early clinical trials shows potential substantial improvement over available treatments. This program provides for timely advice and interactive communications throughout development, as well as intensive involvement from FDA senior management.[1]

**Regenerative Medicine Advanced Therapy designation** (2016) expedites the development and review of regenerative medicines, i.e., cell and gene therapies, for serious conditions where preliminary clinical evidence indicates that the therapy has the potential to address unmet medical needs. This program includes all the benefits of the fast track and breakthrough therapy designation programs, as well as early interactions with FDA, which may be used to discuss potential surrogate or intermediate endpoints to support accelerated approval.[2]

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[1] Food and Drug Administration, Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review. https://www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review

[2] Food and Drug Administration, Regenerative Medicine Advanced Therapy Designation. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/regenerative-medicine-advanced-therapy-designation

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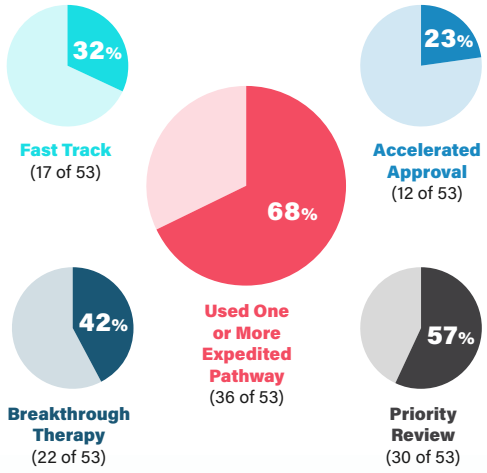
## How Patients with Serious Illnesses Benefit from FDA's Expedited Programs

Expedited programs have been used to bring patients breakthrough treatments for many diseases, including cancer, HIV, cystic fibrosis and many rare diseases. Examples include immunotherapies for treating a variety of cancers, the first-ever gene therapies and the original antiretroviral drugs that continue to help people living with HIV. The FDA's expedited programs have also resulted in the availability of treatments that serve as essential steppingstones toward more seismic shifts in the standard of care.

The accelerated approval pathway, in particular, can be credited with tremendous advances in chronic care and treatment in recent decades. For patients with serious illnesses who cannot afford to wait, the accelerated approval pathway helps ensure effective treatments are available sooner – potentially many years sooner. The pathway provides an avenue for approval before the predicted clinical benefit is confirmed. These so-called "surrogate endpoints" are a necessary part of the development path for certain serious or life-threatening diseases, including rare diseases, which effect small patient populations, are heterogeneous, and variable in their progression.

### 2020'S NOVEL DRUG APPROVALS

**Expedited Review Pathway Usage**




## Potential Solutions to Preserve & Reinforce FDA's Expedited Programs

Patients with serious illnesses deserve the same access to life-improving and life-saving medications as everyone else.

Private and public payers in many states inappropriately restrict access to treatments approved under FDA's expedited programs. **These actions directly undermine the purpose of the expedited programs and Congress' intent by delaying and denying patients urgently needed treatments that are FDA approved and often the only therapy available.** Education is needed on the role and purpose of FDA's expedited programs.

To strengthen FDA's expedited programs and preserve their intent to provide patients with access the medicines they need, Congress should:

- Preserve FDA's expedited programs and reinforce the agency's authority as the gold standard for determining the safety and efficacy of treatments.
- Protect against redundant processes that undermine FDA's authority.
- Guard against policies that threaten to undo decades of progress made in the development of urgently needed medicines that may otherwise not be developed.
- Identify opportunities to enhance the rigor in adhering to the requirements under the expedited programs, including through earlier and consistent interaction.
- Encourage FDA to consider real world evidence to facilitate more meaningful and timely support for confirmatory requirements under the accelerated approval pathway and contribute to a more complete understanding of a drug's clinical benefit and risks.

### Spotlight on Restrictive Policies in Medicaid

**Tennessee's** TennCare Medicaid program is moving forward with a closed formulary that would allow it to decline coverage of certain therapies, including those utilizing expedited programs, and only cover one drug per class.

MassHealth, the Medicaid program in **Massachusetts**, tried to implement a similar program in 2017, excluding drugs they labeled as having "limited or inadequate clinical efficacy." Now it is focused on assessing additional rebates on manufacturers through its Health Policy Commission.

**The Medicaid and CHIP Payment and Access Commission** (MACPAC) has discussed imposing higher rebates for treatments approved under the accelerated approval pathway.

Medicaid and its contracted managed care organizations must be held accountable to their mission of providing healthcare access to America's most vulnerable populations and the requirement that they cover FDA-approved treatments that meet the definition of a "covered outpatient drug."