Biotechnology Innovation Organization

FOOD AND DRUG ADMINISTRATION EXPEDITED APPROVAL PROGRAMS

The Food and Drug Administration (FDA) has established several designations and pathways that grant the drug developer special consideration by the FDA when submitting a therapy for approval. These therapies must be intended to (i) address a serious, unmet medical need or (ii) provide significant improvement over the existing therapy in order to qualify for one of the expedited approval pathways.

All approved therapies, including those receiving expedited approval, must meet the same evidentiary standards required for all FDA approvals. FDA approval is considered the gold standard of approval as the drugs must undergo a rigorous evaluation of safety, quality and effectiveness before they can be marketed. By virtue of the therapy's expedited approval designation, FDA commits greater resources and allows for more frequent engagement with the drug developer to speed up their review timeline.

The goal of expedited approval pathways is to provide safe and effective therapies for serious, unmet medical needs as quickly as possible. It is important to note that certain expedited programs can be combined, in order to most efficiently utilize FDA resources, speed up approvals and deliver needed drugs to patients.

Program Name	Overview	Features	Examples of Indications Treated by Approval Type
Fast Track Designation	Applies to therapies that treat a broad range of serious medical conditions and that (i) address an unmet medical need or (ii) provide a significant improvement in safety or efficacy over existing therapies	• Rolling Review (review at each stage of development vs review of all stages upon completion) • More frequent FDA interaction	Prostate Cancer, Acute Myelogenous Leukemia*, Opioid Withdrawal, Phenylketonuria*, Huntington's Disease*, Beta Thalassemia, Thrombotic Thrombocytopenic Purpura (TTP), Adenosine Deaminase Severe Combined Immune Deficiency (ADA-SCID)
Priority Review Designation	Applies to therapies that provide a significant improvement in safety or efficacy over existing therapies	6-month review time (reduced from 10-month standard review of therapies that are not under an expedited approval program)	Cystic Fibrosis*, Spinal Muscular Atrophy*, Duchenne Muscular Dystrophy*, Multiple Myeloma*, Wet Age-Related Macular Degeneration, Multiple Sclerosis (MS)
Accelerated Approval Pathway	Applies to therapies that (i) treat a broad range of serious medical conditions that address an unmet medical need, (ii) provide a meaningful advantage over available therapies, (iii) approval is based on surrogate or non-traditional endpoints (a marker with evidence that it predicts clinical benefit) likely to be clinically meaningful	• Standard 10-month review • FDA requires Phase 4 (post-approval) studies	Mantle Cell Lymphoma*, Chronic Myelogenous Leukemia (CML)*, HIV*, Chagas Disease*, Fabry's Disease*, Primary Biliary Cirrhosis and Hepatic Fibrosis*
Breakthrough Therapy Designation	Applies to therapies that (i) treat a broad range of serious medical conditions, (ii) address an unmet medical need and (iii) preliminary clinical evidence indicates that the drug may be a substantial improvement over existing therapies	All Fast Track features (see section on Fast Track), but includes earlier and more frequent FDA interaction	Bladder Cancer*, Acute Intermittent Porphyria (AIP)*, Sickle Cell Disease*, Narcolepsy*, Postpartum Depression*, Lambert-Eaton Myasthenic Syndrome (LEMS), Hereditary ATTR Amyloidosis*, Idiopathic Pulmonary Fibrosis (IPF)*
Regenerative Medicines Advanced Therapies Pathway	Applies to cell therapies, therapeutic tissue engineering products, human cell and tissue products, or any combination product using such therapies or products. RMAT products must be (i) intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (ii) the drug has the potential to address unmet medical needs for such disease or condition	Has all the features of breakthrough designation but does not require proof of clinical superiority over existing conditions	At this time, no therapies have been approved via the RMAT designations but there are several promising therapies being developed including a CAR-T therapy for Diffuse Large B-Cell Lymphoma, as well as treatments for Hemophilia A and Stroke

* Denotes an indication with multiple Expedited Approval Designations

Source: https://www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review