FDA DRUG REVIEW PROGRAMS

The FDA has established several programs to support the rare disease drug development process. The four FDA programs below are intended to facilitate and expedite development and review of new drugs to address unmet medical needs in the treatment of a serious or life-threatening condition. However, these designations still require a drug sponsor to gather and provide compelling evidence in support of a drug's safety and efficacy, and this evidence generation takes time.

	WHAT IS IT?	WHEN IS IT USED?
BREAKTHROUGH THERAPY DESIGNATION	Provides additional opportunities to engage with FDA early in the drug development process to strengthen and improve the study design and analysis	Early studies in people indicate that the drug being studied may be substantially better than the currently available therapies being used to treat the condition
FAST TRACK DESIGNATION	Marketing application receives a "rolling review", or review of each section as the marketing application is completed, as well as other benefits	For drugs intended to treat serious conditions and fill an unmet medical need, such as conditions that have a large impact on day-to-day life and will get worse without treatment
ACCELERATED APPROVAL	Allows the possibility of approval based on a surrogate endpoint or an intermediate clinical endpoint instead of a traditional clinical endpoint. Still requires that the sponsor conduct a trial on the traditional clinical endpoint to confirm that the drug has clinical benefit	For serious conditions with no or few treatments
PRIORITY REVIEW	FDA agrees to act on a drug sponsor's application within six months, rather than the ten months required under standard review	For drugs that would provide significant improvements in the safety or effectiveness of treatment, diagnosis, or prevention of serious conditions compared to standard applications

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