CTI CLINICAL TRIAL & CONSULTING
Where Life-Changing Therapies Turn First™

August Newsletter

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FDA Expedited Programs

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CTI offers a full spectrum of clinical trial services with a proactive and integrated approach. On average, CTI plays a role in the development of 10-15% of drugs approved annually by the Food & Drug Administration (FDA) and European Medicines Agency (EMA). In addition, ***CTI has significant experience in each of the FDA's expedited approval programs, typically submitting 8-12 requests each year to the FDA on behalf of clients.*** Let CTI's regulatory experts guide you through these programs:

FDA Expedited Programs

The *New England Journal of Medicine* (NEJM) recently developed and posted online an interactive tool that graphically displays major legislative and regulatory events related to approval of new drugs by the FDA. The tool allows the user to view drug approvals by year and therapeutic category and identify trends in the use of the FDA's expedited approval pathways. You can access the tool by clicking on the following link: http://www.nejm.org/doi/full/10.1056/NEJMp1402114?query=TOC

Since the inception of the FDA in 1937, 1,600 new drugs have been approved for use in the US. The NEJM tool shows that an average of 18 drugs were approved by the FDA each year from 1941 through 1987. However, 4 FDA programs for expedited approval implemented over the past 25 years have helped to increase yearly average new drug approvals to 29. Since 1987, approximately half of all approved drugs benefited from one or more of the FDA's expedited programs. These programs complement one another and serve a common goal to speed the approval of effective treatments for serious conditions. An overview of the programs is provided in the table below:

	Fast Track	Breakthrough Therapy	Priority Review	Accelerated Approval
Type	Designation	Designation	Designation	Approval Pathway
Timing of Application	With or following IND; ideally prior to pre-BLA or pre-NDA meeting	With or following IND; ideally no later than end-of-phase II meeting	With BLA, NDA or efficacy supplement	With BLA or NDA; discuss potential with FDA during drug development
Requirements	Drug is intended to treat a serious condition			
	Nonclinical or clinical data demonstrate potential to address unmet medical need	Preliminary clinical evidence indicating potential for substantial improvement over available therapies on clinically significant endpoint(s)	If approved, would provide a significant improvement in safety or effectiveness over available therapy	Provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate or intermediate clinical endpoint reasonably likely to predict clinical benefit
Features	Opportunity for frequent FDA interaction; rolling review and possible priority review	All Fast Track designation features plus intensive multidisciplinary FDA guidance to increase efficiency of drug development	Decreases the goal of completion of the review and approval decision by 4 months	Approval based on surrogate or intermediate endpoint. Sponsor must conduct additional studies to verify clinical benefit (with studies normally underway before approval.)

U.S. Food and Drug Administration: Guidance for Industry on Expedited Programs for Serious Conditions—Drugs and Biologics. Posted June 2013. Available at http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM358301.pdf. Accessed August 15, 2014.

Overview of FDA Expedited Drug Approval Programs

Fast Track

Fast Track is a designation designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet

CTI Cares Spotlight

Gift of Life Donor Program

Gift of Life Donor Program, the nonprofit organization serving Pennsylvania, New Jersey and Delaware, is responsible for recovering and distributing organs and tissues used in life-saving and life-enhancing transplants.

In addition, Gift of Life coordinates life-enhancing tissue transplants for area residents who are in need of corneas for sight-restoring procedures, as well as skin, tissue and bone to repair injuries.

Nominated by:
Lynn Fallon - President

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medical need. A drug that receives *Fast Track* designation may also be eligible for Accelerated Approval and Priority Review.

Fast Track designation must be requested by the drug company and can be initiated any time during the drug development process. The FDA will review the request and make a decision within sixty days. Upon acceptance, the increased frequency of communication between the drug company and the FDA assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

Since its inception in 1988, 127 drugs with fast track designation have been approved, with the number generally increasing yearly.

Accelerated Approval

It can take many years to fully understand the clinical effect of a new compound. The *Accelerated Approval* pathway allows drugs for serious conditions that fill unmet medical needs to be approved based on surrogate or intermediate clinical endpoints, if there is sufficient scientific support for their predictive power. Further studies will need to be conducted after the drug approval to confirm the drug's effects.

Since inception in 1993, 60 drugs have received accelerated approval.

Priority Review

As part of its commitments in PDUFA V (Prescription Drug User Fee Act), the FDA agreed to specific goals *for* the time needed to review new molecular entity New Drug Applications (NDAs) and original Biologics License Application (BLAs). A *Priority Review* designation directs overall attention and resources to the evaluation of applications for drugs that, if approved, would be significant improvements in the safety or effectiveness of existing approved products for treatment, diagnosis, or prevention of serious conditions. FDA responds to requests for priority review within 45 days after receipt of the NDA or BLA. For drugs granted Priority Review, the goal is to complete the review process within 6 months, rather than the standard 10 months.

Since inception in 1992, 325 approved drugs received priority review.

Breakthrough Therapy

Breakthrough Therapy is a relatively new program that is designed to expedite the development and review of drugs that are intended to treat a serious condition and have preliminary clinical evidence that indicates the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s). Ideally, a Breakthrough Therapy designation request should be submitted by the drug company to the FDA no later than the end-of-phase-2 meetings. FDA responds to requests within sixty days.

A drug that receives Breakthrough Therapy designation is eligible for all Fast Track designation benefits plus additional guidance on efficient drug development programs, organizational commitment and eligibility for rolling review and priority review.

The NEJM graphic does not include drug approvals under the Breakthrough Therapy program, but the FDA website reports 3 such approvals in 2013 and 5 so far in the current year.

Sources:

Kluetz P, Donoghue M. The ASCO Post. FDA Programs to Expedite Drug and Biologic Product Development. Vol. 3, Issue 3. Feb 15, 2014.

http://www.fda.gov/forconsumers/byaudience/forpatientadvocates/speedingaccesstoimportantnewtherapies/ucm128291.htm

For more information:

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Upcoming Meetings We'll be Attending

American College of Clinical Pharmacology
Atlanta, GA - September 14 - 16

Stem Cell Meeting on the Mesa
San Diego, CA - October 6 - 9

Outsourcing in Clinical Trials
Boston, MA - October 7 - 8

New Additions & Promotions at CTI

Brendan Doran, PharmD joins as Research Pharmacist/Study Manager

Grant Ely joins as Business Development Associate

Brian Lawrence joins as Tax and Treasury Manager

Join our Team!! We're looking for individuals to fill these positions:

Clinical Research Associate (US, Spain, UK, Germany, France, Belgium, Poland, Australia, Brazil)

Medical Director (Cincinnati, OH)

Patient Recruiter (Cincinnati, OH)

American Society of Liver Disease (AASLD)
Boston, MA - November 7 - 11

American Society of Nephrology (ASN)
Philadelphia, PA - November 11 - 16

To schedule a meeting with us at one of these, please click here

Matthew Makepeace joins as Administrative Assistant/Receptionist

Michael Wyble joins as Research Associate

Robert Anderson promoted to Senior Systems Administrator

Kim Cohen promoted to Assistant Project Manager

John Green promoted to Information Technology Manager

Janette Montani promoted to Senior Manager, Business Development Operations & Marketing

Lee Muirheid promoted to Senior Programmer/Statistician

Project Coordinator (Cincinnati, OH)

Study Manager (Cincinnati, OH; Philadelphia, PA; Raleigh, NC; San Francisco, CA)

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