FDA/CDER
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Breakthrough Therapies

Inside This Issue

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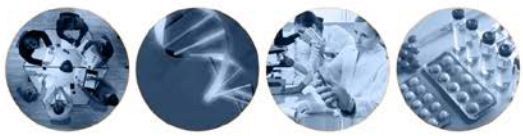
For many years FDA has had programs in place to expedite the development and review of drugs that treat serious and life threatening diseases. Three of these programs are Fast Track, Priority Review and Accelerated Approval. FDA recently established a new program to help expedite the development of new drugs that could potentially offer a substantial improvement over existing therapies for patients with serious or life-threatening diseases who are especially in need of new safe and effective treatments. Section 902 of the recently enacted Food and Drug Administration Safety and Innovation Act (FDASIA) includes a provision that allows sponsors to request that their drug be designated as a "Breakthrough Therapy." FDA recently identified the first therapy to receive this special designation.

What is Breakthrough Therapy Designation? FDASIA defines breakthrough therapy as a drug that is "intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development."

The Breakthrough Therapy Designation is intended to encourage and facilitate the development and review of breakthrough therapies. As for drugs reviewed through FDA's other expedited review programs, drugs that are granted a Breakthrough Therapy Designation still must meet FDA's high standards for safety and effectiveness. All of the programs that expedite development and review are simply approaches intended to make therapeutically important drugs available at an earlier time.

The actions to expedite the development and review of an application that is granted Breakthrough Therapy designation may include:

- holding meetings with the sponsor and the review team throughout the development of the drug
- providing timely advice to, and interactive communication with, the sponsor regarding the development of the drug to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable
- involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review
- assigning a cross-disciplinary project lead for the Food and Drug Administration review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor; and
- taking steps to ensure that the design of the clinical trials is as efficient as practicable, when scientifically appropriate, such as by minimizing the number of patients exposed to a potentially less efficacious treatment.



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How to Request Breakthrough Therapy Designation: FDA is in the process of developing guidance related to this designation. Until guidance is developed, a sponsor should follow the criteria below to make a request for Breakthrough Therapy designation.

A request for Breakthrough Therapy designation should be submitted concurrently with, or as an amendment to an Investigational New Drug Application (IND) with a cover letter, a completed form 1571, and the following information:

- ✓ Identification in the cover letter of the submission as a REQUEST FOR BREAKTHROUGH THERAPY DESIGNATION in bold, uppercase letters.
- ✓ The IND application number, if applicable.
- ✓ The proprietary and/or generic name and established name for drug products; proper name and trade name for biological products.
- ✓ The division or office where the IND is being submitted to or is active.
- ✓ The proposed indication(s) for the product.
- ✓ A brief, but *comprehensive,* summary of information that justifies why the product qualifies for a Breakthrough Therapy designation for the indication being studied, including:
  - Evidence that the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition,
  - Preliminary clinical evidence indicating that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.
- ✓ If applicable, a list of documents previously submitted to the IND that are deemed relevant to the designation request, with reference to submission dates so the documents can be readily located. Copies of such documents can be resubmitted to the FDA as appendices to the designation request (if not too voluminous).

In most cases, this information could be captured in an approximately 10-20 page document.

No later than 60 days after receipt of the submission, a determination will be made to either grant or deny the request for Breakthrough Therapy designation in the form of a designation letter (for requests granted) and a non-designation letter (for requests denied).

Stay tuned for more exciting information from FDA! We will now be publishing these newsletters every other month rather than every month. Suggestions for topics are always welcome!

Until next time,

Renu Lal, Pharm.D.
CDER Small Business Assistance

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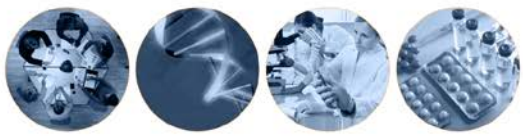
This communication is consistent with 21CFR10.85(k) and constitutes an informal communication that represents our best judgment at this time but does not constitute an advisory opinion, does not necessarily represent the formal position of the FDA, and does not bind or otherwise obligate or commit the agency to the views expressed.



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