GCRC Modified Classification for Rare Disease Research – March 15, 2002

Advances in the treatment of rare diseases are not sufficiently likely to be commercially viable to attract industry-supported development. This is acknowledged by the Orphan Drug Act that specifically permits the Secretary of the Department of Health and Human Services (DHHS) to make grants and/or contracts to assist in defraying the costs of qualified clinical testing of drugs, devices, or foods for rare diseases and conditions.

The NCRR General Clinical Research Centers (GCRC) Program is now following this lead by modifying the GCRC research category classification to facilitate testing of new agents for patients with rare diseases[1]. Consequently, clinical trials of drugs and other candidate therapies may be classified by the local GCRC Advisory Committee (GAC) as category A, instead of D, for drug-company-designed rare disease protocols as described below. (All investigator-initiated trials, designed by a single investigator or a consortium of investigators, are already classified as category A.)

An ad hoc arrangement with the Cystic Fibrosis Foundation's Therapeutics Development Network has led to the conduct of investigator- and drug-company-initiated cystic fibrosis treatment protocols at a number of GCRC sites. This practice is now extended to additional Internet-based Therapeutics Development Networks (TDN)[2] that study other rare diseases. The intent is to enhance the development of effective therapies for patients with rare diseases.

> (1) The phrase "rare disease or condition" refers to any disease or condition that either (A) affects less than 200,000 persons in the United States, or (B) affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales in the United States of such drug or other therapeutic agent.
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> (2) A TDN will be interpreted as a not-for-profit organization constituted to promote advances in treatment for a disease or group of diseases. The organization is required to appoint a group of experts capable of reviewing and prioritizing potential treatment advances on the basis of their merit. The TDN is expected to work with pharmaceutical groups and patient organizations to ensure that new protocols are presented in a form suitable for GAC review.