From: Lee, Bonnie on behalf of OC GCP Questions
Sent: Tuesday, September 20, 2005 2:51 PM
To: [purged]
Subject: RE: IRB activities
Dear [purged],

I apologize for the delay in getting back to you. Starting with orphan drugs, if the drug is not approved for marketing, then in general the rules that apply to investigational drugs also apply to those orphan drugs that are investigational, including IRB review of the study involved. Without more information about the concern/question, it's hard to provide more information. You may want to look at our website which includes the following information:

The term "orphan drug" refers to a product that treats a rare disease affecting fewer than 200,000 Americans. The Orphan Drug Act was signed into law on January 4, 1983. Since the Orphan Drug Act passed, over 100 orphan drugs and biological products have been brought to market.
The intent of the Orphan Drug Act is to stimulate the research, development, and approval of products that treat rare diseases. This mission is accomplished through several mechanisms:
* Sponsors are granted seven years of marketing exclusivity after approval of its orphan drug product.
* Sponsors also are granted tax incentives for clinical research they have undertaken.
* FDA's Office of Orphan Products Development <http://www.fda.gov/orphan/index.htm> coordinates research study design assistance for sponsors of drugs for rare diseases [Notice: This link will take you outside the CDER web site].
* The Office of Orphan Products Development also encourages sponsors to conduct open protocols, allowing patients to be added to ongoing studies.
* Grant funding is available to defray costs of qualified clinical testing expenses incurred in connection with the development of orphan products.

IRBs are supposed to do initial and continuing review of HDEs. (The HDE Q and A at this site may help -- <www.fda.gov/ctlrb/ode/hdeinfo.html>.) There is no protocol, however, for HDEs since they are not clinical studies. The only thing that is some what different about the [purged] is that the company did not have patient labeling when FDA approved it. (Although FDA did not originally require patient labeling, almost all of the HDE holders have it and it looks very much like an informed consent document in the sense that it lists the potential risks, benefits, etc.) They were one of the earlier HDEs and did not have such labeling when they first went to market. They may it now though as we think a lot of the IRBs kept asking them for it.

I hope this information is helpful to you.

Sincerely,

Bonnie

Bonnie M. Lee
Associate Director for Human Subject Protection Policy
Good Clinical Practice Program, FDA
This communication does not constitute a written advisory opinion under 21 CFR 10.85, but rather is an informal communication under 21 CFR 10.85(k) which represents the best judgment of the employee providing it. This information does not necessarily represent the formal position of FDA, and does not bind or otherwise obligate or commit the agency to the views expressed.

-----Original Message-----
From: [purged]
Sent: Wednesday, September 07, 2005 2:00 PM
To: OC GCP Questions
Subject: IRB activities

While conducting an inspection at the [purged] the chairman of the IRB had questioned the IRB's requirement for protocol review and continued review of Humanitarian Device Exemption (HDE) and orphan drugs. The IRB currently has a protocol for [purged], which was presented and approved in March 2002. I believe the orphan drug requirement was a general interest question as there are no current protocols reviewed for such drugs by this IRB.

Please respond with comment. I will also provide the chairman with the GCP contact website for further discussion.

Thank you,

[purged]