From: OC GCP Questions
To: [redacted]
Subject: RE: Question: Siblings in Study for Rare Disease in Pediatric Population
Date: Wednesday, October 01, 2014 12:16:04 PM

[redacted],

You raise several good questions that can only be answered by having a full understanding of the protocol in question and the medical condition being evaluated. I recommend the sponsor (presumably [redacted]) contact the review division within the FDA responsible for the study about your concerns. The review division will request an internal FDA Ethics consult if they believe it is needed.

I hope this information is helpful to you. If further assistance is needed, please feel free to contact us once again at the official GCP mailbox, gcp.questions@fda.hhs.gov

Thank you,

Kevin
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301-796-3707

From: [redacted]
Sent: Tuesday, September 30, 2014 9:27 AM
To: OC GCP Questions
Subject: Question: Siblings in Study for Rare Disease in Pediatric Population

My question refers to siblings in a study for a rare disease in the pediatric population. Statistically it is a problem for the study as it is a randomized study with a subjective outcome - the measure of itch based on the caregiver's observation. The main issues are protection of the blind as well as independence of observations in the study. Statistically we would say no- neither child can be enrolled. This is difficult because this is a rare disease- so it is hard to find patients, and also there is no other treatment for these kids so it is an ethical issue. Thus we have a balance between the statistical issues and the rare disease/ethical issues. These trials are also quite small (24 pts) but will hopefully be used for registration. One idea suggested was to have the two siblings enroll in the trial but be randomized to the same treatment. The analysis would have a sensitivity analysis for the impact of this. Question: Given that this is a rare disease, how would the agency view this option? Are there other options for siblings in this setting- ie rare disease, subjective outcome

measure?

Regards,
[redacted]