

NIH/TRND NPC Cyclodextrin Clinical Trial ~ Update

International Conference Call Held on Friday, May 3rd, 2013

Addendum

Community Questions and NIH TRND Team Responses

Speakers/Presenters:

Dr. Daniel Ory, MD

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Call Hosted by the NNPDF

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Mom to Ty, age 16 (NPC)

Nadine M. Hill

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May 16th, 2013

NPC Community inquiry: *Does anybody have any information regarding the FDA hold letter specifying what the NIH needs to do to get off the hold? Denny Porter said the hold letter had to be sent to the NIH within seven days - so it should have arrived by May 8.*

Dr. Forbes “Denny” Porter responds:

1. We need to provide them with a description of what we did to try to determine the cause of the problem. Should not be an issue at all and basically have this drafted. Would be a critical issue if we were proposing to continue to use Ommaya’s. In other words have we identified the problem and revised the approach in order to decrease the risk of another occurrence. Also, gives them in one document information on drug sterility testing etc that will still apply.

2. Revised protocol and consent document.

Since it is on an official hold, they will have a 30 day clock to review the response.