

NIH/TRND NPC Cyclodextrin Clinical Trial ~ Update

**International Conference Call
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Speakers/Presenters:

Dr. Daniel Ory, MD

NNPDF Scientific Advisory Board (SAB) Chair
Professor of Medicine, Cell Biology & Physiology
Washington University School of Medicine
St. Louis, Missouri

Dr. Forbes D. Porter, MD, Ph.D.

NNPDF Scientific Advisory Board (SAB) Member
Senior Investigator, Section on Molecular Dysmorphology, Program in Developmental Endocrinology and Genetics
National Institute of Child Health & Human Development
National Institutes of Health
Bethesda, Maryland

Nicole Yanjanin, MSN, CPNP

NNPDF Director-at-Large
Clinical Research Nurse
National Institutes of Health NPC Clinic
Bethesda, Maryland

Jonathan Jacoby

NPC Parent Advocate

Karen Quandt, R.N., M.S.N.

Chair, National Niemann-Pick Disease Foundation
Mom to Ty, age 16 (NPC)

Nadine M. Hill

NNPDF Executive Director
National Niemann-Pick Disease Foundation
Fort Atkinson, Wisconsin

NOTE: There is a brief glossary of frequently used terms in this transcript at the end of the document.

Call began at 10:30am CDT with Nadine Hill of the NNPDF:

Nadine Hill: I would like to welcome everyone to this call being sponsored by the National Niemann-Pick Disease Foundation for our International Niemann-Pick Type C community. We are going to be receiving updates today from members of the National Institute of Health TRND Team that are doing work at Bethesda Maryland at the National Institute of Health with regards to the Cyclodextrin Clinical Trial.

Joining us today are Dr. Denny Porter, Dr. Dan Ory, and Nicole Yanjanin who works together with our clients and our patients at the NIH. In addition, Karen Quandt, who is the board chair of the National Niemann-Pick Disease Foundation, as well as, Jonathan Jacoby who is our patient advocate and a parent advocate and he is going to be spearheading the call. As we get started this morning we just want to indicate to you that we anticipate the call will be 45 minutes in length. We have indicated it on our web site, where you can submit questions to the moderators to be reviewed via e-mail. That e-mail address is listed on our web site, and it is also, I'll say it quickly, nichdnpc1@mail.nih.gov. So those will be reviewed and then forwarded on to our presenter, Jonathan Jacoby. At the end of the call, if we have gone through all the e-mails responses and discussion in regards to the items presented if we have time we will then offer our guests the ability to unmute their phone lines and be able to submit questions to our speakers.

At this time, in addition to welcoming you all to this call, I would like to turn this over to Jonathan Jacoby.

Jonathan Jacoby: Hi everybody. As Nadine said we're going to spend most of the time on this call just hearing an update from Denny Porter and Dan Ory about the trial. As you'll hear, there is mostly information about what has happened, and information about where we want to go. It's probably true that we have more questions than answers at this point. And so I want to just assure everybody that within the next number weeks we will not only get out as many answers as we can, but we will, when we're ready, try to have another call or another way of facilitating questions and answers so that we're as transparent in sharing as possible.

So with that I just want to thank the NNPDF, first of all, for hosting this and organizing it, and especially thank Dr. Porter and Dr. Ory for all of their hard work. And I'll hand it over to you Denny.

Dr. Forbes Denny Porter: Okay. So, we asked Jonathan and the NNPDF to help organize this call so that we could update the community on the status of trial. Unfortunately, it's not the best news, in that we have had to pause the trial at the NIH. I think fundamentally this can happen in research trials. We have to remember that research trials are experiments, they are not therapies. Especially early on, safety of the patient has to be paramount. What has happened in the NIH trial is that we've had 2 out of 3 patients having an infection of the Ommaya reservoir. The infection is from a bacteria that normally lives on the skin, and is normally associated with acne. Although infection of the Ommaya reservoir is what we call an "anticipated event", it *does* happen, and we recognize that the numbers are low, we have an extremely high frequency and we're actually concerned about the specific identity of this bacteria.

We're also aware of the patient outside the trial who had bleeding after placement of the Ommaya reservoir. I think trying to work through these issues, it has raised the concern that NPC patients may be at greater risk than we anticipated with the placement of the Ommaya reservoir. With these issues, what we elected to do as a research team, was to pause the protocol at that point. We've been re-evaluating the problems as we go, as they were occurring, but we reached the point where we had too many problems and not enough answers.

The NICHD IRB supported the decision to pause the trial. In reality, I'd think if the research team had not paused the trial, the IRB would've stopped the trial. Again, because safety is what needs to be paramount.

Dr. Denny Porter (cont.):

We had a conversation with the FDA on Wednesday of this week. They were updated with regards to the various problems and they have put us officially on a clinical hold. This, obviously, we can understand is extremely frustrating to the NPC community, extremely disappointing. We, as a research team, share that feeling. I think Jonathan mentioned, you know, the status update was not only what has happened, but also where we're going.

As frustrating as having to pause the trial because of hitting problems, the information that we did get is promising. So there actually is some good news that came out of the initial attempt to try to use Cyclodextrin through the Ommaya reservoir. First good news is the problems that we have encountered are very unlikely to be drug related. They appear to be device related. The second thing is that the biomarker data looks promising. The third thing is that we got good PK data from these first three patients. So we have some tools to go forward.

Our goal is to figure out how to move forward, and what we're working on right now is trying to separate the device and the drug. Probably the most direct way to do that is actually to step back and consider giving the drug by lumbar intrathecal injections, and that's what has been done in children up to this date. It's probably, and when we set this up, wasn't the most attractive way forward. It's not without its own set of risks. It's not without its own set of problems, and difficulties. Some of which the Ommaya reservoir would have solved. We won't get as much data as we would've gotten if we were able to use the Ommaya reservoir, but we believe we can get the critical data that we need.

So that's where we are in terms of thinking how to move forward. I have already initiated discussion with the NICHD IRB. I suspect that they will be favorable on allowing us to pursue that direction. We also raised this possibility with the FDA on our teleconference call on Wednesday (5/1/13). Officially what the FDA will do is they will send us a hold letter, and they have to send it within 7 days. We don't have it now; they don't have to send it until next Wednesday. In that hold letter they will specify what we need to do to get off the hold. So, that's absolutely critical in defining the path forward, and we'll see exactly what concerns they have and how much work we'll need to do to get this moving forward.

We did raise the question on Wednesday on whether our pre-clinical animal testing would be sufficient to support lumbar intrathecal delivery. And my interpretation of the conversation was that they would likely view that as being sufficient. Obviously, we will have to wait on the official letter. But that was extremely promising, because obviously if we had to go back and repeat toxicology testing in the animals, that would take significant time. They did raise concerns about the bleeding risk and whether it was possibly related to hydroxypropyl-beta-cyclodextrin. It's not clear whether that will become part of their hold letter, and what we would have to do to try to address that if that continues to be a concern.

So, I think the fundamental thing is that we're committed to trying to move this forward; putting plans in place. We obviously have to rewrite the protocol, we have to work out mechanics of getting it done here, but we have a good idea of how to do that. We will have to see the FDA letter, because that will really dictate the areas where they raised a concern and really dictate what we will have to do to move this forward. But everybody that's been working on this project over the last couple years is committed to moving it forward. And, I think, the fact that we have some promising data from the biomarkers really gives us the suggestion that we have the tools. Where hypothetical when we started the trial will actually work and help us sort out the issues we might face with intrathecal delivery.

So the goal is to move it forward. We've hit a major problem, there's no way around stating that. Right now, on everybody's mind, the question is, how long will this take? I hope it's as short a time as possible. We will work to make it as short as possible. We have unknowns right now, primarily what will the FDA require us to do that make answering that question definitively not possible at this point and time.

Dr. Denny Porter (cont.):

I think once we get that, once we put together a clear plan and think it through that is something we can update the community on. And really, that's the status of the trial and sort of a summary from my standpoint.

Jonathan Jacoby: Thank you Denny. Dan, do you want to add anything?

Dr. Dan Ory: That was a great summary that Denny provided. I did want to just add a little information in terms of the feedback we were getting from the FDA. Which is that on the call with the FDA, Denny and his team outlined in great detail the steps that have been taken, were taken, during the trial to address the issues as they arose. So, it isn't that we just waited until this time and re-assessed the data. When there was an infection, or colonization that was noted, Denny and his team immediately began to review the standard operating procedures and put into place very, very stringent protocols in order to minimize the possibility that we would see another infection. And, as well, obviously involved epidemiologists, ID specialists, and neurosurgeons at NIH to look at this. So, in many ways, everything that we could think of was being done to try to minimize the occurrence of these infections. And I think that the conclusion that we reached, although based on a very small number of patients, would suggest that there might be something unique in terms of potentially the indwelling device that was used and the NPC1 biology. Obviously more work would need to be done to sort this out, but it's just something that I think we need to keep in mind. Because the way in which the Ommayas were being placed and being monitored and cared for was using the most stringent protocols, you would not have anticipated, as Denny said, the rate of infection that we did observe.

I do think also that the FDA certainly indicated to us that they appreciated the degree of diligence that the NIH team had given to this problem and, as well, they were very pleased that we're communicating with the community this information so that we maintain, as you indicated Jonathan, as much transparency as possible.

Jonathan Jacoby: Okay, thanks. There are a few questions that have come in. Let me put them out there and a couple that sort of go together.

Participant Question: *"The acne type bacteria common in the general population, is there any relationship with NPC kids?"*

Dr. Denny Porter: Okay, so the bacterium is called Propionibacterium acnes. It normally lives in your pores and we could wipe anyone's skin and find it. It's an organism that you will find infecting shunts, Ommaya reservoirs and items of surgical implantation. The question is very good, is it different in NPC1 or NPC patients. There's a possibility. The bacteria lives in the pores and is really lipophilic. It likes lipids and as we all know lipids are perturbed in NPC patients. Also, there's not much literature on it, but when you look at it the bacteria actually uses an enzyme called Sphingomyelinase in its pathology. Sphingomyelinase and Sphingomyelin can be altered in NPC. That's actually the primary defect in NP-A and NP-B, and it's one the reasons why these diseases originally got classified together. So, we don't know if this is the reason, I mean this would just be hypothesis, one could speculate that perhaps there is a higher density of this particular bacteria on NPC1 patients compared to other patients. We just don't know. Those are hypotheses.

Obviously they're researching ones one would think about pursuing. It would not impact directly where we go in the future, because I think our concern is within in-dwelling catheters and to try to separate the device from the drug so we can get the data that we need to move forward on the drug. But there are definitely questions to be asked.

I wouldn't be concerned about this particular bacteria because we don't see infections with it in NPC patients outside of this context where we put in an indwelling catheter. This bacteria is very indolent. Meaning it doesn't cause somebody to get sick very fast, even if they have an infection, but it can be hard to clear if you have a foreign body involved and that's what we had with the Ommaya reservoirs.

Dr. Denny Porter (cont.):

We actually tried to clear the first case, because that child was not sick, that child had no symptoms, and that's what we refer to as colonization. In the second case, it was picked up and the child had some symptoms, so that would've been an infection. In that setting you don't even try to clear, you need to get rid of the foreign device.

Jonathan Jacoby: Thanks Denny. Another question:

Participant Question: *“My understanding of the Cyclodextrin function would have been acting as a carrier to remove lipids build up by removing it through cell wall. Is that correct? If so, is the by-product lipid Cyclodextrin product removed or metabolized by body?”*

Dr. Denny Porter: I could address that Denny. For purposes of treating NPC, Cholesterol removal is actually not occurring with the cyclodextrin. That is we're treating at concentrations low enough we don't actually extract the cholesterol stores from the cells.

Rather the way the cholesterol or the cyclodextrin is actually working within the neurons is to cause a redistribution of the cholesterol. It's normally stored within the lysosomes. The cyclodextrin, in a way we don't fully understand, somehow engages those membranes and acts as a trigger to release the stored cholesterol. It's that stored cholesterol, when it is released, that goes to other parts of the cell. That is how we are able to detect whether or not the cyclodextrin is acting.

So, the other part of the question was whether or not the cyclodextrin was being metabolized, and the answer is no. Cyclodextrin does not get metabolized within the body. The cyclodextrin moves into the tissues, and then it moves out of the tissues and it goes into the blood stream and 98% of the cyclodextrin is filtered by the kidneys and excreted in the urine.

Jonathan Jacoby: I'm thinking then a related question is:

Participant Question: *“How does this relate to the good data you have? Does it mean the kids that actually got the cyclodextrin; you saw some positive results right away? What can you tell us about that?”*

Dr. Denny Porter: Okay, so. First of all, what we're looking at are biomarkers when I talked about the data that we got. Related to what Dan was talking about, was this redistribution of cholesterol. We had hypothesized that this redistribution would result in the production of a specific oxysterol. And that oxysterol would be a marker that the redistribution that the cyclodextrin induced the redistribution in the neurons. And that's what we observed. So, we were able to confirm in the kids what we've also seen in the mouse and in the cat. Now with the kids and actually confirm it at a very low dose. We did not expect to see something at the dose the FDA asked us to start with. What we saw was what I referred to as a biochemical response. This trial was not set up to look for a clinical response. To really make those conclusions one has to do it under very strict conditions, where you don't have placebo affects influencing either the patients, the parents or the physician trying to ascertain whether a drug has an effect or not. In this trial, even though we are looking to get information on how outcome measures focused on clinical effects might be applied to a future trial, it's not designed in a way to conclude that we had positive physical benefits.

Be that as it may, we did design the trial to see if we could have the biochemical effect.

Jonathan Jacoby: So Denny, related to that- it's good that all these questions are related to each other.

Participant Question: *“What does that suggest about the dosage that might be needed for lumbar punctures, for IT delivery, given that you originally decided to go with the Ommaya reservoir? And if you might also comment on if this has any change in your thinking about IV delivery?”*

Dr. Denny Porter: Okay, so, first of all with regards to the intrathecal... when we were considering how to do this, there were a number of things that moved us away from intrathecal towards the Ommaya. Some of it I sort of touched on earlier. One, the mechanics were potentially easier. The ability to really monitor the cerebral spinal fluid was much better, so we could get more information. We were delivering the drug exactly to where we wanted it, or much closer to where we wanted it, than if we were using the lumbar intrathecal.

When we first started this, a major question was if we use the lumbar intrathecal, would the drug make it all the way up the spinal cord to the brain where we wanted it to go? If we had a negative result, we wouldn't be able to interpret whether it was our biomarkers weren't any good or the drug just wasn't getting to where we want. So part of the good news of having the response that we had was, that the biomarkers looks like it is working like we predicted. So we can now use that as a tool with regards to the intrathecal.

As to what the dose would be, I don't know, and that will be one of our first questions. Given an equivalent dose intrathecal, do we see an equivalent response? My guess is no, but that's just a guess, but we have the tools to actually answer the question. Is a response happening? Was there an attempt? Don't you see a response? But we have the tools to try and take it apart, which is significant. The other part of the question is does it change my opinion on IV therapy? Absolutely not.

Jonathan Jacoby: Could you just give a little bit more of an explanation of why?

Dr. Denny Porter: It's still a fundamental issue of getting enough across the blood brain barrier. I don't believe that you can deliver IV cyclodextrin in sufficient quantities to truly get the effects we want to have, and I think that you just incur risks without doing what we want to do, which is to get the drug into the central nervous system to the neurons.

Jonathan Jacoby: Okay.

Participant Question: *"Is the criteria for being in Phase 1 going to change as result of this?"*

Dr. Denny Porter: So, the inclusion/exclusion criteria are important. Part of their reason is to decrease the possibility of having adverse events. You know, we can't anticipate all the adverse events or what their frequency will be. I will have to go back and actually sit with this and think through every one of them in the new context, but my first response to that question is, probably not. Again, because their purpose is safety. We've just seen the effects of adverse events can have on a clinical trial, and you don't want to have adverse events that aren't related to the trial itself. Especially with small numbers they can really confuse things.

So, without having the chance of actually thinking through every one of them, in the context that we've moved to lumbar intrathecal in general, they won't change, though there will be a few added, such as if you had spinal anomalies we wouldn't do it. If you have an infection of the skin over the lumbar region, you wouldn't do it. So there's some that are added, other than ones related directly to an Ommaya. They are unlikely to change and that because, again, safety is paramount, especially early on.

We understand how frustrating it can be to parents whose children don't make the inclusion/exclusion criteria, but if we have the opportunity to do ten individuals and then something happens, we can say it's at low frequency and unlikely related to the drug. When you have small numbers and something happens in the first few patients you really have a problem, and that's part of what we're dealing with now.

Jonathan Jacoby:

Participant Question: *"Is there any possibility that the infection was related to miglustat? Do you know if those patients were on miglustat?"*

Dr. Denny Porter: Would I assign the infection to miglustat? I don't know of any biological reason to do that. My bias at this point is that it's an organism that can cause problems with surgical implants and there are some aspects of this particular organism biology that are concerning that it might be related to NPC1. It is, I think, the question should be expanded. It is a reason why we formally do not allow other medications, other supplements. We do allow for miglustat, that's the one exception. We don't allow for other supplements in this trial.

They just become confounders in trying to interpret the situation. So unless somebody has to be on a medication for a medical indication, a standard medication, it's not allowed. That's one aspect of trying to be as safe as possible and trying to be able to interpret situations or problems when you run into them.

Jonathan Jacoby: Thanks Denny. I just want to remind everybody of the e-mail address, which is, nichdnpc1@mail.nih.gov. We have two more questions and we have a few minutes after that if there are any other questions.

Participant Question: *“Can you say anything about experience with Ommayas in other countries or where there might have been patients who used it?”*

Dr. Denny Porter: So, when we hit this problem I did contact the physicians in Brazil and they've had one out of two Ommayas get infected. It's actually a different organism, a different bacteria but they had to remove one out of two. That's really the information I have on outside this study basically.

Jonathan Jacoby: And I did see the patient in Japan did not have an infection, and of course, what are the others? The two sisters did not have an infection.

Dan, maybe just a follow up on your answer to the question about redistribution of cyclodextrin.

Participant Question: *“If it does act to redistribute, does it eventually return and begin building again in the lysosomes? The cholesterol?”*

Dr. Dan Ory: So, the answer is probably yes. We have some studies underway where we're doing repeated doses of the cyclodextrin in the mouse & cat models and are then able to look at the cholesterol biomarkers in those settings. What it really does look like though is that the re-accumulation is not as fast as we thought it might be. It really does appear that, certainly in some of the animal models where we're treating every two weeks, that we have sufficiently depleted the cholesterol and the lysosomes and that the cyclodextrin seems to have a fairly long acting effect. Certainly it can go beyond a week and perhaps as long as two weeks. Therefore, when we give repeated doses of the cyclodextrin we can see that there is a change in some of the biomarker measures indicating that the storage is not re-accumulating very quickly.

More studies clearly have to be done, but I think that's actually very hopeful, because it may give us some clues as to what type of dosing frequency will be needed. One could certainly envision a situation where you might treat NPC not unlike the way you actually do a chemotherapy where you undergo a rapid induction phase with many treatments. Perhaps spread out over a two week period and then perhaps go to a maintenance phase where you can receive your doses on a monthly basis. That certainly is possible.

Jonathan Jacoby: Thank you. I do want to remind everybody that the only data that can be used for this trial is the data directly from the trial. So while there is information about patients in other places, that data isn't allowed to be incorporated into this specific trial. Although it may be useful for other purposes.

Any other questions that anyone wants to e-mail now? We'll wait another second and if not then I want to remind everybody that the e-mail address is going to be around for the duration, if there are any questions that anybody else has, feel free to e-mail them. Every question will be answered as soon as there's news from the FDA, and then it will be sent to you. I just want to check to see whether Nadine, or Dan, or Denny or Nicole have any other questions or comments?

Nadine Hill: What I could tell you at this point is that if you don't have any questions being sent to you via e-mail, that we could indicate to our participants that they could unmute their phone line and ask a verbal question to the team if they have something that perhaps wasn't addressed. Just before I go ahead and tell you how to follow those instructions, I want to remind everyone that we will be supplying a recorded link to the call so that you can re-listen to what Dr. Porter & Dr. Ory presented for us this morning, as well as a written transcript including all the follow-up questions that should come into the e-mail, will be included in that transcribed report as well. So, at this time, if you have any questions, you can unmute your individual phone line by hitting *6 on your keypad. That is * 6.

And then Jonathan, I guess we'll wait a little bit for people to come online if they should have something, and then you may have to work a little bit if we have duplicate calls where people talk over one another.

Jonathan Jacoby: Okay, we have about 5 more minutes. So if anybody has a question just * 6 and ask it and then hopefully Denny or Dan will be able to answer it. Although I must say that such a good job of answering whatever questions there are so far, that I'm not sure there's a lot more that can be discussed right now. Oh, here's one.

Participant Question: *"How much cyclodextrin do the doctors think will make it to the brain via the IT?"*

I think actually you answered that question Denny, but if you want to say another word about it.

Dr. Denny Porter: Yes, so, it's to be determined. I think in parallel with trying to get the clinical trial back on track, we've discussed doing experiments in the cat model that would look at delivering it lumbar and seeing how much gets to the brain and compare that to the high intrathecal that is normally done there. We also have a monkey experiment that we had been planning here and we're changing that experiment. Part of the goal would be to compare high intrathecal versus lumbar intrathecal in the monkey.

In the animal models, putting in the Ommaya reservoirs is difficult and not practical and that's why we've used the high intrathecal there. High intrathecal is not really practical in humans. It's just some anatomical differences. So we're working to compare in the animal models. And as I said, we don't know, but I think we have the tools to determine that. To determine what might be equivalent to the 50 mg that we saw with the intraventricular delivery.

We've got lots of questions we still have to answer. Repetitive dosing. We obviously only got to the first dose, so our plan would've been to increase in subsequent patients to ask if 100 mg would do better. Where do we max out the effect? So those questions still remain. I think we can still get them answered. You know, what's the optimal dose? What's the dosing frequency? What happens on repetitive dosing? All those questions are absolutely critical to taking forward to a second trial that will be focused on clinical symptoms. We don't have enough patients to guess and just start a trial and hope we guessed right.

Jonathan Jacoby: We're going to hear a lot of background noise right now, so I want to thank everybody. I want to remind everybody that, again, to send in questions and you'll be able to send them questions after you see the next update as well. We're going to set up a more systematic way of doing this over the next month or so, so that we'll have constant communication available throughout the course of the trial. For updates on particular patients who may be doing their own individual IND trial, please go to their individual web sites so you can get them. Again, many thanks to everybody for making this happen so quickly and many prayers and much positive energies for all of our children.

One more question right at the close.

Participant Question: *“Is the cyclo pump being considered Denny?”*

Dr. Denny Porter: So, I think our major issue right now is that we feel we had a problem with infection with the device. That makes me concerned about any implanted device right now. So the goal really was to step back and say, let’s focus on getting the information we need on the drug and separate the drug from the device. That’s why we say we need to put the emphasis on getting the information on the drug. That will move forward.

Caller Question: *“Has this affected the natural health study at all?”*

Dr. Denny Porter: Sorry, the natural history study?

Caller Question: *“Right, the natural history study. Has that been affected by this?”*

Dr. Denny Porter: So no, that’s still open. It affects it perhaps in a way that we didn’t want it affected; we sort of wound the natural history study down just because of manpower issues. We need to see how long it will take us to get this up and going. Hopefully, it’s short and we will have a gap in time that we’ll fill in with the natural history. So I don’t think it will be affected at all. The natural history actually helps us, because we’ve got significant data on over a hundred LPs that we’ve done on individuals with NPC1. So we can truly put numbers to the safety issues and adverse event rate.

Caller Question: *“I had one follow up question. You mention redistributed lipids that the cyclodextrin cause those to leave the lysosomes and be redistributed with the possibility of coming back later. What is the long term impact of those redistributed lipids? Are they eventually get removed? Do they stay there? I guess what’s the end game for that aspect of it?”*

Dr. Dan Ory: Cholesterol itself gets redistributed in the cell, although what does happen is that some of that cholesterol is getting converted into a more soluble form of cholesterol, an oxysterol. It’s released from the cell and that’s part of the normal physiology of cholesterol turnover in the brain.

Caller Response: *“Okay. That was my concern is that if it’s just re-releasing the same stuff then it’s gonna come back. But, that makes sense.”*

Dr. Dan Ory: You ask a good question and the other question is if there are other lipids that are accumulated within the lysosome; there are ones we call sphingolipids, or sometimes you’ve heard to them referred to as ganglioside, a type of sphingolipids. Those are inside the lysosome. Those are released. We can tell that based upon on some beautiful neuropathology work that Steve Walkley has done. And so, the fate of that is uncertain.

Jonathan Jacoby: There is a lot of background noise now; I’m just going to say two more things. One is that when we essentially get a more systematic way of sharing this information we will also include information about questions like the one just asked so that people understand the mechanism and be able to have a very easy access to resources about the disease and the mechanism of this particular treatment. The e-mail address again is nichdnp1@mail.nih.gov. It’s also on the NNPDF web site.

I hope everybody has a great weekend. Thanks to all and let’s hope that this thing gets back on track as quickly as possible. Goodbye everybody, thank you very much.

End of Call.

Sponsors Note: All at the NNPDF would like to offer a great note of thanks to our speakers today, Dr. Denny Porter, Dr. Dan Ory, Nicole Yanjanin and Jonathan Jacoby for their assistance is helping the foundation to share this important information with the wider NPC community. Their time and talent are appreciated more than we can express. In addition, we offer a note of appreciation to our NPC community ~

We **WILL** Persevere in our Quest for a Cure.

Glossary:

CSF	Cerebral Spinal Fluid
FDA	United States Food and Drug Administration
ICV	Intracerebroventricular
IND	Investigational New Drug
IRB	Institutional Review Board
IT	Intrathecal
NICHD	National Institute of Child Health and Human Development
NIH	National Institutes of Health
NPC	Niemann-Pick Disease Type C Disease
TRND	Therapeutics for Rare and Neglected Diseases

Note: This call was hosted by the National Niemann-Pick Disease Foundation. If you have any questions pertaining to the material presented here ~ please feel free to contact us.

National Niemann-Pick Disease Foundation
401 Madison Avenue, Suite B; Post Office Box 49
Fort Atkinson, WI 53538-0049
Phone: 1-920-563-0930
Fax: 1-920-563-0931
E-mail: nmpdf@nmpdf.org
Web site: www.nmpdf.org

