Niemann Pick Disease Clinical Trials

This is an exciting and enviable time for the NPD community with several clinical trials currently in the news. It can be a confusing time too as families try to decipher all of the available information to understand what the trials are about and whether their loved ones a) can qualify/be eligible, b) want to be involved and c) is available to them geographically.

Below is a document that explains what clinical trials are and provides a list of potential questions to ask as part of your research into the trial.

Disclaimer

The "51 and Done" is a committee composed strictly of parents dedicated to providing information and knowledge to the Niemann Pick Type C community on ALL upcoming and future clinical trials. The intent is to ensure parents have objective and relevant information and knowledge to help them decide on the path to take for their loved ones affected by Niemann Pick. Please note that the “51 and Done” committee does not engage in the practice of medicine or claim to have medical knowledge. The main purpose of this committee is to provide information and knowledge from publicly available sources.

All material contained in this document is for educational and informative purposes only. It is not intended to substitute medical or professional services nor replace the relationship that exists between patients, their physicians and other health care providers. No action, treatment, independent entities or persons are endorsed and all information is provided without warranty. Any questions should be referred to your physicians and in particular, any changes in treatment or care should be made in conjunction with your medical team.

Since information on the critical trials are under constant revision, it is recommended that you refer to the links provided below for the latest and more complete information about the trial including updates on inclusion/exclusion criteria for trial participation.

Clinical Trials

What Is a Clinical Trial?

It is important to understand that clinical studies are experiments. It typically involves conducting research on human volunteers/participants with the intent to determine if a specific drug or therapy is effective. The two main types of clinical trials are: a) interventional studies, and b) observational studies.

In a clinical trial, participants receive specific interventions, such as a drug, according to the protocol created by the investigators that guides the trial. The intent is to determine the safety and efficacy of the intervention/observation by monitoring/measuring certain outcomes in the participants.

Clinical trials are sometimes described by phases as described below:

- **Phase 1:** The goal in a phase I trial is to determine whether the drug is safe and to find out the effective dose.
- **Phase 2:** In Phase 2, the drug is given to a large group of people to see if it is effective and to further evaluate its safety.
- **Phase 3:** The drug is given to larger groups of people to confirm its effectiveness, monitor side effects, compare the effects of newer drugs with current treatments, and collect information that will allow the drug or treatment to be used safely.
- **Phase 4:** Studies are done after the drug or treatment has been approved and marketed to gather information on the drug's effect in various populations and any side effects associated with long-term use.

Who sponsors/conducts the Clinical Trial?

A clinical trial is typically sponsored or funded by a pharmaceutical company or an institution such as a medical research institution, the National Institutes of Health (NIH) or the European Commission (EC).

A clinical trial is typically conducted by a Principal Investigator (PI) along with a research team who assists with the study. The research team includes doctors, nurses and other health care professionals.

Who can participate in a clinical trial?

The protocol, which guides the study, lists the eligibility requirements to participate in the study. These are listed under the inclusion criteria and exclusion criteria. Examples of eligibility requirements would typically include age of the participant, stage of disease, previous treatment and other medical conditions.

To get the latest information on the protocol and the eligibility requirements for the current trials in NPD, follow the links below:

For NPB

- For pediatric patients
  https://www.clinicaltrials.gov/ct2/show/study/NCT02292654?term=NCT02292654&rank=1#contacts
- For adult patients
  https://www.clinicaltrials.gov/ct2/show/NCT02004691?term=acid+sphingomyelinase&rank=5

For NPC

- NIH/Vorinostat -
  https://clinicaltrials.gov/ct2/show/NCT02124083?term=vorinostat+niemann&rank=1
- Orphazyme/ Arimoclomol
  https://clinicaltrials.gov/ct2/show/NCT02435030?term=orphazyme&rank=1
- Vtesse/VTS-270 (Cyclodextrin)
  https://clinicaltrials.gov/ct2/show/NCT02534844?term=vtesse&rank=1

What is an Informed Consent (IC)?

IC is the process in which researchers provide interested participants with information about the clinical study including the risks and potential benefits of the clinical study. The idea is to provide as much information as needed in order for the participant to decide whether he or she wants to enroll into the clinical trial. Note that providing consent is not a contract. A participant can withdraw from the study at any time.

Additional questions to ask/consider

It is important to learn as much as possible about any clinical trial so that you can make an informed decision on whether to participate or not. Below is a potential list of questions to consider when doing research or to ask the research team about the study.

- **What is being studied?**
- **Why do researchers believe the intervention being tested might be effective? What are the primary and secondary outcomes? (e.g. improved swallowing, better gait, increase in life expectancy)**
- **If neurological symptoms are involved, what will the drug's expected effect be?**
- **If systemic symptoms are involved, what will the drug's expected effect be?**
- **How do the possible risks, side effects, and benefits of this trial compare with those of my/my child's current treatment?**
- **What kind of prior research has been done to prove the efficacy of the drug?**
- **Has the treatment been studied in several different animal models, and especially in one that is thought to mirror how it will act in humans**
- **Are the risks of using this drug known? Are the potential risks understood?**
- **Is the proposed treatment part of a formal study (clinical trial)? If so, what is the current phase of the clinical trial? What is the status of the IND and EMA submissions and regulatory approvals of your program?**
- **If I/my child benefit from the intervention, will I/my child be allowed to continue receiving it after the trial ends? If so, who will pay for it and for how long?**

- **Other questions to ask:**
    - What will I/ my child be required to do if I/ my child decide to participate?
    - Will it be a placebo controlled trial?
    - What tests and procedures are involved?
    - How often will I/ my child have to visit the hospital or clinic?
    - When visiting the clinical site, how long will the process take?
    - Will hospitalization be required?
    - How long will the study last?
    - Who will pay for my participation/treatment?
    - Will I/ my child be reimbursed for other expenses?
    - What type of long-term follow-up care is part of this trial?
    - Will results of the study be provided to me?
    - Who will oversee my/ my child's medical care while I/ my child is participating in the trial?
- Has this drug been used previously to treat other diseases? For example, prior to its approval to treat NPC, Zavesca had been approved for Gaucher disease.
- What is the dosage?
- What are the side effects?
- How will the drug be administered?
- Has a plan for follow-up monitoring been defined for participants after the treatment?

- At which locations will the study be conducted?
- What clinical experience has there been with your product to date?
- What is the current phase of the clinical trial? (Note: The further along the clinical trial, the more information is available about dosage and efficacy.)
- What assurance will I/my child have that the trial results will be published promptly, regardless of trial outcome?
- Is the involved pharmaceutical company/medical research institution experienced in running a clinical trial?
- Is the involved pharmaceutical company/medical research institute financially capable to support the trial to the end?

Remember: You can check www.clinicaltrials.gov to determine if it is a formal clinical trial in the U.S.)