

OVERVIEW OF CLINICAL TRIALS

There are multiple clinical trials that are currently underway for Pompe disease. Some of them are for enzyme replacement therapies, some are observational studies, and some look at combinations of treatments/drugs. In the near future it is likely that we will see gene therapy clinical trials for Pompe disease as well, possibly from multiple companies or research institutions.

A full list of all clinical trials for Pompe can be found by going to www.clinicaltrials.gov and searching for “Pompe.”

Because there are so many different trials, which is unique for a rare disease like Pompe, it is important to understand the clinical trial process, the trials phases, and other basic information. If you are interested in participating in clinical trials, the AMDA suggests that you review this information, and then discuss any questions you may have with your doctor. Please note that this is just a short overview of clinical trials in general and some questions you may want to discuss with your doctor.

What are the 4 Clinical Trial Phases?

- **Phase I:**
 - Includes a small number of participants;
 - Primary purpose is to look at the safety of the proposed treatment and safe dosing
- **Phase II:**
 - Primary purpose is to study the safety and effectiveness of the proposed treatment
 - It can last from several months to several years
 - “Most phase II studies are randomized trials where one group of patients receives the experimental drug, while a second ‘control’ group receives a standard treatment or placebo.” (<http://www.centerwatch.com/clinical-trials/overview.aspx>)
- **Phase III:**
 - Primary purposes are to look at the safety and effectiveness of the proposed treatment in a larger population than Phase II.
- **Phase IV:**
 - These are post-marketing studies that occur after the FDA has approved a treatment.
 - Can be used to study the long-term effectiveness of a treatment

What questions might I want to ask my doctor?

- What Phase is the trial in? Phase I, II, III, or IV?
- What is the purpose of the study? Observational? Interventional (i.e. treatment will be provided)?
- What will my responsibilities be if I participate? Travel requirements? Testing? Etc?

- Are there any possible side effects I should know about?
- What risks and benefits should I consider?
- How long will the study last?

What happens when the trial/study is over?

“After a clinical trial is completed, the researchers carefully examine information collected during the study before making decisions about the meaning of the findings and about the need for further testing. After a phase I or II trial, the researchers decide whether to move on to the next phase or to stop testing the treatment or procedure because it was unsafe or not effective. When a phase III trial is completed, the researchers examine the information and decide whether the results have medical importance.

Results from clinical trials are often published in peer-reviewed scientific journals.” (Excerpt from <https://www.nih.gov/health-information/nih-clinical-research-trials-you/basics>).

Resources for Learning More:

National Institute of Health (NIH): <https://www.nih.gov/health-information/nih-clinical-research-trials-you>

National Institute of Health (NIH) Infographic: <https://www.ncbi.nlm.nih.gov/books/NBK209903/>

Food and Drug Administration (FDA): <https://www.fda.gov/forpatients/approvals/drugs/ucm405622.htm>

Center Watch: <http://www.centerwatch.com/clinical-trials/overview.aspx>