



Trofinetide Phase 3 Clinical Trial Q&A Last updated: 2/2/18

About trofinetide

Q1. How does trofinetide work?

A1. Trofinetide aims to correct four hallmark pathological features of many central nervous system disorders: inflammation, over-activation of microglia, dysfunction of synapses and reduced levels of IGF-1. By simultaneously targeting multiple processes, trofinetide works to restore natural balance to brain function. Watch Neuren's [video](#) about trofinetide for more information about these four areas.

Q2. What are the potential risks and benefits?

A2. Trofinetide demonstrated high safety, very low risk, in the two Phase 2 trials. The benefits seen in Phase 2 were far reaching and affected a number of the challenges seen in Rett syndrome. Each patient may experience different benefits; as a group many of the challenges in Rett syndrome will show improvement. See [results](#) of the pediatric study.

Q3. What symptoms will trofinetide alleviate?

A3. See [results](#) of the pediatric study.

Q4. What form is the medication?

A4. It is a liquid and taken orally or via gastrostomy tube.

Q5. What is needed to get FDA approval?

A5. This study design has two primary outcome measures. If these measures are found to show significant improvement in Rett syndrome during treatment with trofinetide, then Neuren Pharmaceuticals and the FDA will discuss the data. If they reach agreement about the results, then Neuren and the FDA will work to determine dosing and delivery of the drug for use in Rett syndrome.

About the Phase 2 trofinetide trials

Q1. What occurred in the trofinetide Phase 2A and 2B trials?

A1. The adult Phase 2 ran from 2013 through 2014 and was a 40-day double-blind low-dose drug study (then called *NNZ-2566*) that included 67 females with Rett syndrome between the ages of 16 and 45, and was conducted at 3 clinical trial sites in the U.S.

The pediatric Phase 2 during 2016 was an 11-week double-blind higher-dose drug study (renamed *trofinetide*) that included 82 females with Rett syndrome between the ages of 5 and 15, and was conducted at 12 clinical trial sites in the U.S.

About the Phase 3 trofinetide trial

Q1. When will the trial start and end? Is my child eligible to participate? Are boys eligible? How do I enroll? Will the trial require overnight stays and how often? What will happen during the study? Where are the clinical trial sites?

A1. Each drug company develops inclusion and exclusion criteria as well as trial design and protocols for their clinical trial, often taking into account input from the FDA and the patient community. The Phase 3 information is not yet finalized for publication. Rett syndrome.org will work with Neuren Pharmaceutical to develop a website to answer all of these questions and more. When the answers are available and published on this website, we will announce it for all to follow and explore.

Q2. Will a participant be eligible if they have previously participated in a drug trial, or are currently enrolled in a clinical drug trial?

A2. This is a good question that will be answered once the enrollment criteria are published. Past participation in one trial usually does not exclude future participation in a different clinical trial. Usually, there is a “washout period” required between clinical trials. Please contact the clinic team of the studies you’re interested in to ask what their guidelines are for planning purposes. Rarely can one patient be simultaneously enrolled in more than one trial at a time; otherwise scientific outcomes would be confounded. We will know more once the specific criteria are made available for Neuren’s Phase 3 clinical trial.

Q3. Will participants know if they are receiving the drug or placebo?

A3. This trial is a double-blind study meaning that neither the patient nor the doctor knows whether the participant is on placebo or drug. When the trial is over that information can be made available.

Q4. Will a family be compensated for participating during the trial?

A4. Not to our knowledge.

Q5. Is there help available to pay for travel expenses?

A5. We are hopeful that there will be partners helping families with travel assistance.

Q6. Is there housing at the site in the event a family has to stay for some period of time?

A6. We do not yet know the study locations, so we cannot comment on the types of housing available in the event overnight stays are required. Most hospitals engaged in clinical trials have housing and transportation fact sheets produced for their participating families.

Q7. If the participant wishes to continue on trofinetide after the trial, will the drug be available? A7.
We do not know at this time. Neuren will provide the answer.

Q8. Which private Rett syndrome organizations have funded clinical trials of trofinetide?

A8. Rettsyndrome.org is the only Rett organization that has directly sponsored the clinical trial of trofinetide for treatment of Rett syndrome, with thanks to our donor community who made this possible. Rettland has partnered to provide travel support for participants in the past.

About the impact on a participant

Q1. What if I have questions during the study, who do I ask?

A1. Site Study coordinator

Q2. Can participants leave the study once it has started?

A2. Yes

Q3. How is a team of physicians informed about a participant's care during the clinical trial?

A3. Tell them you are participating and give them the contact number of the site study coordinator.

Q4. What if a participant's medications, school, or therapy programs need to change during the course of the trial? A4. Talk to the study coordinator and inform them of the change.

Q5. Will a participant's daily medication combination need to change or stop during the course of the clinical trial? A5. Usually not, this will be clarified once the protocol is published.