Webinar Questions 7-8-13

1. Now that the generic Riluzole has come to market, will the trials be written to include patients receiving the generic drug and not only Rilutek?

   Clinical trials allow use of riluzole. This will include the brand name version and the generic.

2. I hear that NP001 will be starting a new trial phase very soon. What do you know?

   There is interest in another trial. We have not heard any information on timing or details on protocol. We will inquire again though with Neuraltus and post any new information we learn.

3. Will mesenchymal Stem Cells administered intravenously will be of any benefit? Brainstorm in Israel use intramuscular approach for initial stages and intrathecal for advanced stages, whereas Neuralstem goes for intaventricular.

   There is no data that intravenous Mesenchymal stem cells would be of benefit. Neuralstem product is administered directly into the spinal cord.

4. It was my understanding that it is not yet known how C9 orf72 does its damage? I also thought antisense works if the issue is a sort of toxicity? If I am correct on the previous statements if the c9 problem is something else is there a real danger that c9 antisense just won't work?

   Antisense technology should decrease formation of protein from mutation. We are all hopeful that this approach will be effective and safe. We don’t know for sure of course, but there are ongoing studies in the laboratory and plans to bring this to people with c9orf72 ALS.
5. How will trial regroup/proceed after placebo error that Cytokinetics announced & how such problems can be avoided in future?

   We need to learn more about what happened. This type of event is fortunately very uncommon.

6. What's a typical expense per patient for a Ph II #ALS trial? I've heard $20K. Is that even close?

   The cost depends on the intervention. Participants do not cover the costs for being part of a study. Some studies, the per patient costs is for example 5000. For others it could be up to 100,000. The costs include for example, surgery (for Neuralstem), the study drug, the laboratory testing, and the time of personnel involved in study.

7. What therapies in stages of clinical trials do you think most promising? What will be the first therapy to come? When do you expect that the therapy will be available?

   All the therapies currently in clinical trials are hopeful. We don’t know which might work better than another. Most of the therapies are now in early phase trials (phase 1 or 2).

8. Can I get the test? My mother died from ALS and I need to know if my children will do the same. Thank you.

   The best approach is to meet with a genetic counselor to review your family history and discuss the probability of inheriting ALS. If you mother was the only person in your family with ALS, the chance is very low. A genetic counselor can help guide you through the decision about testing.

9. Are there any charitable organizations that can help people internationally get to the US for treatments?

   I am not aware of any that offer this. Sometimes, local people might help.