Q: You mentioned that there was one dramatically positive result to the Neuralstem trial -- could you elaborate on this & tell us what follow up is being done?

A: We don't know why this one person improved. One possibility is that it was from the immunosuppressive treatments that were administered with the stem cells; another is that it was from stem cells and the third is that it had nothing to do with either the immunosuppressive drugs or the stem cells. We are about to start a trial of just the immunosuppression drugs; this study will be at MGH, Emory and U Mass.

Q: Time magazine just reported on a new faster research approach for cancer. Can that approach be used for ALS?

A: Team Science is very important in ALS; open sharing of data, collaboration across fields, laboratories and institutions will help move science forward faster. This is already happening in ALS.

Q: Have there been any attempts that you know of to cull data from PatientsLikeMe?

A: Yes, Patientslike me have published papers from the data they collect.

Q: Should siblings of "sporadic als" patients be getting genetic testing, based on your comments around the blurring of the lines between "familial and sporadic"?

A: I do not think siblings of sporadic als patients should get genetic testing. I do think however, that we are moving towards era of genetic testing, at least for c9orf72 mutations, in all patients with sporadic als. If someone is positive for a genetic mutation associated with als, then discussions for siblings with genetic counselor about testing would be warranted.

Q: I am interested in joining clinical trial of Brainstorm. Are screening of clinical trial done firstly by documents? What documents are required? Or are the screening process done by direct diagnosis immediately? Will the planned clinical trial of Brain storm in USA continue the ongoing clinical trial in Israel? Or will it repeat the phase 2a from the beginning?

A: The details of US study have not been determined. It would likely not be a repeat study from Israel but more a continuation of the drug development plan. The goals would be continued safety and preliminary efficacy. The criteria for entering the study are not known at this time.
Q: I was put on intravenous immunoglobulin (ivig) for 1 year because my EMG showed conduction block. More recent EMG showed psw fibs. Why is it so hard for a diagnosis?

A: Early on in the illness, it can sometimes be hard to be 100% confident on the diagnosis of ALS. The EMG for example in someone with bulbar onset can often be normal the first few months or year. The field does need to develop better methods to diagnose sooner, so therapies can be started sooner. There are several efforts underway to improve diagnostic timelines.

Q: Why are we subject to be called the throw away people just because were older in prognosis? What are you doing so that the older patients can get into the stem cell trials?

A: There are studies where patients anywhere in the course of the disease are eligible (Cytokinetics for example). There are other studies where there are scientific reasons to test therapy earlier in the disease course. As mentioned on the webinar, it may be that certain treatments work better in different phases of the disorder.

Q: What do you know of any holistic health procedures which may help the general health of ALS patients? What about detoxifying the body with iodine, making an alkaline versus acidic body through intake of baking soda and manganese skin ointment? Is there any knowledge of longer survival with a body free of lead, florine and mercury?

A: I am not aware of any data that demonstrates that these approaches work in ALS.

Q: In the Air Force during the 1970's I worked in close proximity to nuclear weapons for 4 years. Is there any connection there?

A: This is not known.

Q: Are there any other inhibitors of macrophages/monocytes in addition to NP001 in clinical development?

A: I do not think so.

Q: Our diagnosis is PBP with no extremity involvement yet. Do we need to wait for treatment since it isn't ALS yet? Also with having PBP are there clinical trials available?

A: Nuedexta is a potential trial for people with only bulbar signs.

Q: There are many promising treatments in the pipeline, but how many of these will help PALS who have been diagnosed more than 5 years ago?

A: We don't know if the treatments in the pipeline will work, nor if they will be better in different phases of the illness. These are important questions and need to be looked at in patients in all stages.
Q: We are seeing growing numbers of veterans with ALS in our hospital. Is there a known link with the military, ie. vaccines, chemical exposure etc. Is there a higher incidence rate in the military vs civilian population?

A: The risk is higher in the military. We do not know why, though there are ongoing studies to address this risk.

Q: Are you able to comment on the lawsuit between Knopp and Biogen Idec whether we'll see dexpramipexole again?

A: I don't have any information on this.

Q: Please tell me if the persons with ALS have access to the drug Nuedexta. Also please tell me if there are clinical trials for persons with ALS in stage 3 almost 4.

A: Nuedexta is a marketed drug (available by prescription). It is marketed for the treatment of excess emotions associated with ALS. Cytokinetic study is available for people independent of how long they have had the illness.