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Good morning, my name is Nicholas Di Prospero and I am a clinical fellow with Kurt Fischbeck. Through my time with him, I've been studying mostly Huntington's Disease in a model system, which we're hoping share some features of the pathology with Kennedy's, in hopes of designing therapeutic interventions. And, we're also spending time evaluating the possibilities of doing some clinical trials at NIH for Kennedy's Disease in the future.

So, Dr. Fischbeck, Dr. Sumner, and myself have talked at some length about doing a clinical trial here in the United States for Kennedy's Disease, and in preparation for that, we're working on designing a survey, similar to one that the Kennedy's Disease Association has already put out. I was trying to get a good idea about the clinical issues, along with other features that are shared that may not be completely recognized in the current literature regarding Kennedy's Disease.

In that sense, when you design a clinical trial, you want to actually cast a very wide net so that we can not just look at things that we can measure objectively such as muscle testing and nerve conduction, but things that are also subjectively important to a patient's quality of life standards. When you actually go to look at the effects of a drug, some things may change disproportionately in terms of time, so you may notice that you may have a better quality of life in terms of being able to button your shirt before you're able to lift ten pounds more with your leg. So, we're trying to cast a broad net when we do the clinical trial in the hopes of gaining a lot more information. Therefore, we're trying to bring out, or develop as I said, a survey form, which we're hoping we can get out sometime in the next few months.

Additionally, we'll keep in very close contact with Dr. Sobue's group, in following their trial to see how they're making out in terms of progress and discuss the possible therapeutic modalities in terms of drugs that we'd like to pursue. Hopefully Dr. Sobue will be able to, give you a little better information about how his work's progressing as well. (Applause)

Question from Audience:

(Inaudible). We've had (inaudible) people to our survey and we are all volunteer. It's a tedious thing to get this information collated. We are not finished with it with but there's a wealth of critical information there, but frankly (inaudible) we'll need help. Is there a way that, through NIH or through some funding group, that we could get funded, if you will, and we could hire somebody to do some of the things that we need to do to assist you.

Di Prospero

Funding issues are difficult ones. Most funding comes from grant sources, but what you're, what you're describing is really my day job. My day job is involved in trying to pursue really the essence of what you are doing here. So the idea of, of not just getting information but analyzing it and collating it, as you're saying, is something that, individual researchers can do. As far as doing it yourself, I don't necessarily know about how you could go about getting grant money from something like the NIH. What it essentially comes down to is that researchers like ourselves, myself, or others basically apply for additional funds saying that this is part of their objectives in their research design that they want to look at. So what is great is that taking some of the observations from the small survey that's been done here analyzing the data and refining it. I know that there's been a number of, of respondents to the KDA website. Hopefully we can cast a very large net and be able to solicit information from even more people. But again I think, it falls really to the research personnel to really kind of pursue what you're asking.

Question:

We just don't have the time to do this. In the interest of time and sharing (inaudible) would that be of benefit?

Di Prospero:

Sharing information is wonderful. However, to do anything under the auspices of a clinical trial, we're asked to go through standards to provide patient protection, which is done through what's called the Institution Regulatory Board, or Review Board, in other words an IRB. This review process was instituted by federal law for protection of patients and individual subjects. So basically sharing some information with us in an anonymous fashion is well and good, but for us to actually go about and communicate that information in a publication or something to the worldwide community of scientists, has to be done under certain stringent guidelines. So, we have to actually go through many processes in order to bring forth a kind of survey, but having a starting point from the information you gathered is a great boon.

Audience:

Nick and I were just talking about that at the break that our survey is almost complete as far as just getting information tallied. We're not extrapolating any correlations or trying to make any judgments at this time, but just to get the raw data over to them and then he can look through our survey, he said he may even be able to utilize portions of it in his own survey; he's got to develop it towards their clinical trial, in the way that (inaudible).

Question:

When your survey is developed who will be asked to complete?

Di Prospero:

Basically, anyone who has confirmed Kennedy's disease and is willing to participate. The great thing is the KDA is a wonderful resource for patients and researchers. The KDA may advertise the fact that we are doing this study on their website and we would also solicit through the NIH sponsored advertisements and website basically saying we have developed this survey to help us get a better understanding of Kennedy's disease. It is then up individuals to contact us and express an interest. The procedure, would be to present further information and provide the patients with a consent form that one would sign indicating that he agrees to be part of this survey. In this way, a formal consent should explain the study so everybody fully understands what's involved.