**Supplementary Material**

**Language included in the survey provided to participants via a web-based format.**

We invite you to participate in a research study conducted by investigators at The University of Pennsylvania. The purpose of this study is to evaluate the knowledge and perception of individuals towards gene therapy for Huntington’s Disease (HD). This survey is meant to obtain information about an individual’s beliefs and attitudes towards gene therapy for HD. We are inviting you to be part of this study because you have HD, you are at risk to develop HD or you take care of an individual with HD. If you agree to participate, we would like you to complete the following survey. You are free to end your participation at any time. It will take approximately 15 minutes to complete the survey. We will not collect your name or any identifying information about you or the person in your care. It will not be possible to link you to your response on the survey. Taking part in this research study is completely voluntary. If you do not wish to participate in this study, do not continue beyond this point. If you wish to quit at any time, please close the survey window. If you change your mind later and wish to have your response deleted from study records, please contact Tanya Bardakjian at Tanya.bardakjian@uphs.upenn.edu. You will not receive any financial compensation for taking this survey. If you have any questions about the rights of research subjects, please contact the Office of Regulatory Affairs at the University of Pennsylvania by calling 215-898-2614. Thank you very much for your consideration of this research study. If you would like to proceed to the survey at this time, please click the option to do so below. If you would prefer not to participate, please close the browser window.

Are you at risk for developing Huntington Disease: Yes No Unsure (Select One)

Did you test positive for the Huntington Disease gene: Yes No (Select One)

Do you have a diagnosis of Huntington Disease: Yes No (Select One)

If yes, at what age did you develop symptoms? \_\_\_

How old are you now?\_\_\_\_

Are you: Male Female (Select One)

What is your highest level of education? (Select One) GED High School College Graduate School

**Gene therapy clinical trials are research programs being developed to test the safety of gene therapy as a treatment for HD.**

How likely are you to participate in gene therapy research trials?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to cure your HD?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to slow the progression of HD?

1.Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to stop the progression of HD?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials knowing there will be no clinical benefit to you but will definitely help future generations?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials knowing there will be no clinical benefit to you but might help future generation?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials if there is no risk to you but limited improvement of your condition?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials if there was a high risk to you and a higher chance of improvement?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

**You have been diagnosed with Huntington’s Disease but your symptoms do not affect your daily activities. You are working and can still do everything you want with your family and friends. Your doctor indicates you might have 5-10 years before you have to stop working and require a great amount of help.**

How likely are you to participate in gene therapy research trials to cure your HD if treatment was taking a pill once a day?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to cure your HD if treatment was an IV infusion in the arm requiring a visit to the hospital/clinic once a week?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to cure your HD if treatment was an IV infusion in the arm requiring a visit to the hospital/clinic once a month?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to cure your HD if treatment was an IV infusion in the arm requiring a visit to the hospital/clinic once a year?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials to cure your HD if treatment was an injection into the spinal fluid requiring a visit to the hospital/clinic once a month?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

**Injection into the brain (please consider the following)**

The procedure offered is a one-time injection of "gene therapy" into a specific part of your brain. The goal of this "gene therapy" is to slow down or stop the disease. This has been done before in mice and monkeys and it was found to be helpful and safe. But, it has not been tested in humans so we do not know if the procedure is safe in humans. The goal of this study, done for the first time in humans, is to see if the “gene therapy” is safe. If you agree to participate in this study, you will be under general anesthesia for several hours. A small hole will be drilled into your skull and a fine needle will be inserted into one area in your brain. Once there, the gene therapy will be injected through the needle for 2 hours. Once your brain has enough gene therapy, the procedure will be over. Before and after the procedure for one year, you will have to complete other tests including physical examinations, brain MRI (3 times) and spinal tap (3 times).

 There are two options:

1) You can get the gene therapy injection into one side of your brain. In this case, you might not benefit as much from the procedure. On the other hand, if the gene therapy is not safe, it will only affect one side of your brain.

2) You can get the gene therapy injected into both sides of your brain at the same time. In this case, you may benefit more from the procedure. However, if the gene therapy is not safe, it may cause more harm since it is in both side of your brain.

How likely are you to participate in gene therapy research trials if the treatment was injected into one side of the brain?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

How likely are you to participate in gene therapy research trials if the treatment was injected into both sides of the brain which would include two injections at the same time?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

Sometimes in research, we need to measure how individuals respond to treatment compared to another group that does not receive the same treatment. We use something called a placebo, or an inactive treatment to measure this. The most common example is when some participants receive an active drug and some receive a “sugar pill”. In these trials, half of the participants are randomly selected to receive the treatment we believe to be beneficial to the patient and the other half receives the placebo.

How likely are you to participate in gene therapy research trials if there was a chance you would not receive the treatment?

1. Very Unlikely

2. Unlikely

3. Likely

4. Very Likely

**How important is the following in your decision to participate in gene therapy research trials?**

Helping my children who are at risk

1. Not important at all

2. Somewhat important,

3. Very important

Improving my quality of life

1. Not important at all

2. Somewhat important,

3. Very important

Risk to myself

1. Not important at all

2. Somewhat important,

3. Very important

Curing HD

1. Not important at all

2. Somewhat important,

3. Very important

Helping science

1. Not important at all

2. Somewhat important,

3. Very important

**What do you believe is most important to researchers doing gene therapy research trials?**

Safety of participants

1. Not important at all

2. Somewhat important,

3. Very important

Financial gain

1. Not important at all

2. Somewhat important,

3. Very important

Helping patients

1. Not important at all

2. Somewhat important,

3. Very important

Advancing science

1. Not important at all

2. Somewhat important,

3. Very important

Helping their career

1. Not important at all

2. Somewhat important,

3. Very important