



Boston Children's Hospital

We are currently seeking participants for a focus group!

Research Study Title: *Characterization of Central Manifestations in Niemann Pick Type C Disease*

What is the purpose of the focus group? *We are aiming to perform a study involving adult patients with Niemann-Pick Type C (NPC) to better understand causes of neurological dysfunction in this illness. The focus group is meant to help us learn about the neurological and psychiatric symptoms that affect NPC patients and their families as well as determine what elements of their disease are important for patients to understand more about or find treatments for. Additionally, we hope that these conversations can help us assess feasibility of performing certain studies in patients with NPC.*

The investigators will ask you and the other people in the focus group to use only first names during the group session. They will also ask group members not to tell anyone outside the group what was said during the focus group discussion. However, the investigators cannot guarantee that everyone will keep the discussions private.

Who can participate? *Patients with NPC and their family members or caregivers. All participants must be over the age of 18 years.*

Where is the focus group being conducted? *Virtually, via secure Zoom video and audio conferencing.*

What do I have to do if I'm in the focus group? *We will ask questions about your experience with NPC and ask for your insight into the feasibility of our study. Information you wish to share only with the study team can be communicated outside of the focus group and will be kept confidential. Furthermore, sharing of personal health information is not needed to be part of the focus group.*

What is the time commitment for the focus group? *We anticipate that the conversation will take approximately 45-60 minutes.*

What are the benefits of the focus group? *In addition to helping the study staff learn more about aspects of living with NPC and assessing feasibility of the study, we hope that the focus group will be an opportunity for participants to interact with other members of the NPC community.*

What are the risks of the focus group? *There is a risk that the identity of anyone participating in the focus group will become known to other participants and the investigators carrying out the focus group.*

What will I receive from participating? *Compensation or other incentives will not be offered for participating in the focus group, but is provided for the in-person studies we are conducting.*



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This research is being conducted by: *Jaymin Upadhyay, PhD and Walla Al-Hertani, MD*

For additional information regarding this focus group and study, please contact:

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