



Children's Hospital Boston  
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[www.childrenshospital.org](http://www.childrenshospital.org)



**HARVARD MEDICAL  
SCHOOL**  
TEACHING HOSPITAL

Dear \_\_\_\_\_,

You/your family member are being asked to participate in a research study to evaluate the safety and effectiveness of treatment of NPC1 with hydroxypropyl- $\beta$ -cyclodextrin (HP- $\beta$ -CD). HP- $\beta$ -CD is an investigational (experimental) drug that is administered through an intrathecal (IT) lumbar puncture (LP), which means the medicine will be given through a needle inserted through your back into your spinal canal (the space containing spinal fluid below your spinal cord) and mixed with the spinal fluid. This study will investigate if treatment with HP- $\beta$ -CD can halt or improve your/your child's symptoms of NPC1 and identify the optimal dose for effective treatment.

You/your family member are being asked to participate in this study because you/ your child are between the ages of 4 and 21 and have a confirmed diagnosis of NPC1.

This study involves testing an investigational drug. This means the drug has not yet been approved by the Food and Drug Administration (FDA). Information from this research will help determine what effect hydroxypropyl- $\beta$ -cyclodextrin has on patients with NPC1 and whether the drug should be approved by the FDA in the future.

This study is being sponsored by Vtesse, a pharmaceutical company located in Maryland. Boston Children's Hospital has been asked to participate in this study along with other hospitals in the US and worldwide. Up to 8 patients will be enrolled in this study at Boston Children's Hospital and approximately 51 patients will be enrolled between all sites participating.

Olaf Bodamer, MD PhD will be the Principal Investigator for the study at Boston Children's Hospital. He will work with multiple other doctors, nurses and study team members including anesthesiologists and audiologists. The study will take place in various units in Boston Children's Hospital.

If you/ your family member decide to take part in this study, you/ your child will need to visit Boston Children's Hospital for treatment every 2 weeks for one year to have the investigational drug administered through LP. Typical visits will last 2 days and will include physical and neurological exams with Dr. Bodamer, blood tests, and urine samples. Certain visits will also include hearing tests and other fine and gross motor assessments. On the weeks you do not come to the hospital, we will call you to check how you are feeling.

You do not have to pay to be in this study. All of your travel expenses will be covered including flights, car service, taxi, and lodging, and meals for you as the participant along with two companions (for the first two months) and one companion after that, will be reimbursed on the days that you come to Boston Children's Hospital.

We will contact you in the next few weeks to see if you are interested in participating in this study or to answer any questions you may have about it. If you would not like us to contact you or if we have the wrong contact information, please return the form in the postage-paid envelope provided.

Please do not hesitate to contact me via e-mail should you have any additional questions.

Thank you for your consideration of this study.

Sincerely,

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