

A Research Study About Magnetic Resonance Imaging (MRI) and Krabbe Disease

Diffusion Tensor Imaging (DTI) as a Tool To Identify Infants Who Have Krabbe Disease and are in Need of Urgent Treatment

Purpose

The purpose of this research study is to determine if a magnetic resonance imaging (MRI) technique can be used to detect subtle changes in the brain of young children who have been found to have a low level of an enzyme that is associated with Krabbe disease. As newborn screening for Krabbe disease becomes widespread, this tool can aid physicians and families with decisions about treatment and follow-up care.

Procedures

Participants will have a comprehensive developmental evaluation and an MRI scan three times over a two-year period. During the visit, the developmental evaluation will take two to three hours, depending on the child's age, and the MRI will take about one hour.

Reimbursement

Participants will receive \$150 for completion of the MRI and developmental evaluation for each visit. The study will pay for travel, including airfare for two parents and one child, a one-night hotel stay, and food reimbursement.

Risks

There are risks associated with any procedure. If the child is unable to fall asleep naturally for the test, he or she may be given sedation medicine. Any risks associated with these procedures will be explained in detail prior to participation in the study.

Investigators

Principal Investigator

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Co-Investigators

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This research study is sponsored by National Institutes of Health (NINDS 1R01NS061965-01), and has been approved by the Institutional Review Board of the University of Pittsburgh.

If you would like your child to participate in this study, please contact our study coordinator.

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Further information is also available at our website at www.chp.edu/ndrd.

This research study is being conducted by the Program for the Study of Neurodevelopment in Rare Disorders (NDRD) at Children's Hospital of Pittsburgh of UPMC.

**Program for the Study of Neurodevelopment
in Rare Disorders**



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