



THE UNIVERSITY
of NORTH CAROLINA
at CHAPEL HILL

CLINICAL CENTER FOR THE
STUDY OF DEVELOPMENT
AND LEARNING

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This is an Invitation to Participate in a Study of the Natural History of Late Infantile MLD

You are receiving this information because your child was diagnosed with Metachromatic Leukodystrophy (MLD).

The purpose of this study is to understand what happens to the neurodevelopment of children with MLD. This research will help understand if future treatments like enzyme replacement infusions or cord blood transplantation are effective in improving or stabilizing the disease progression. If you would like to participate, your child will have a comprehensive neurodevelopmental evaluation by a clinician who is an expert in evaluating and treating children with MLD. The work up will include 2 magnetic resonance imaging (MRI) scans of the brain (one at baseline and one at 6-months) and other neurological tests necessary to understand how the disease is progressing. The studies will be done at the Program for Neurodevelopmental Function in Rare Disorders (NFRD) at the University of North Carolina at Chapel Hill.

The study sponsor, Shire Human Genetics Inc, will pay for travel, hotel accommodations, partial food reimbursement and transportation expenses for both parents and the child. Your child will need to come three times during a period of 6-months. At each visit your child's development will be followed and recommendations about therapy will be provided. The evaluations will include a physical, a neurological evaluation and assessments of cognitive, language and motor development. In addition you will receive counseling about feeding/eating, sleep and best interventions/ therapies to improve the quality of life and your child's overall development.

This study was approved by the Biomedical IRB at the University of North Carolina at Chapel Hill
Study# 07-1239

To learn more about this study, please call or e-mail:

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