



Opportunities to Participate in NF Research

"Variability of Individuals and Families with NF-1"

Department of Genetics, University of Alabama, Birmingham, AL

Tricia Page 1-866-822-4362

e-mail: nfstudies@genetics.uab.edu

Bruce Korf, M.D., Ph.D. in collaboration with the Molecular Neurogenetics Unit at Massachusetts General Hospital in Boston is investigating the variability of individuals and families with NF-1. They are focusing on defining factors that contribute to differences between affected individuals in the number of neurofibromas (tumors). Men and women age 19 years and older diagnosed with NF-1 may be eligible to participate in this study. Study participants will complete a questionnaire, have anonymous photographs taken, and some may have a blood sample drawn. The time commitment is approximately 40 minutes. There is no cost for the participation and no compensation.

"Bone Density Study in NF-1"

Baylor College of Medicine, Houston, TX

Alyssa Tran at (832) 822-4264

e-mail: alyssat@bcm.tmc.edu

Dr. Brendan Lee is looking at the density or thickness of the bones of people with NF-1. Adults and children with NF-1 can take part in the study. A painless DXA scan which exposes the body to less radiation than a regular chest x-ray will be performed free of charge.

"Novel Imaging Modalities for Plexiform Neurofibromas"

Children's Hospital of Philadelphia, Philadelphia, PA (only)

Michael Fisher, M.D. at (215) 590-3129

Gihan Tenakoon, M.D. (neurology) (215) 590-1000

Dr. Fisher will be evaluating two biologically distinct methods for assessing plexiform neurofibromas – 18FDG PET and MR perfusion – to determine if they can be used as markers to distinguish benign from

malignant tumors and to predict responses to therapy for lesions that have persisted in size following treatment. Individuals with NF-1 who are younger than 25 years may be eligible to participate in the study.

"Clinical Trial and Molecular Analysis of Tibial Pseuarthrosis"

University of Utah School of Medicine, Salt Lake City, Utah
David Viskochil, M.D., Ph.D. at (801) 581-8943
e-mail: David.Viskochil@hsc.utah.edu

"Molecular Analysis of Peripheral Nerve Sheath Tumors"

University of Utah School of Medicine, Salt Lake City, Utah
David Viskochil, M.D., Ph.D. at (801) 581-8943
e-mail: David.Viskochil@hsc.utah.edu

"NF-1 & Reading Difficulties@ (Ages 6 to 14)"

Kennedy Krieger Institute, Baltimore, Maryland
Jessica Abel at (443) 923-9268
e-mail: abelj@kennedykrieger.org

Laurie E. Cutting, Ph.D. is conducting the study of children with NF-1 between the ages of 6 and 14 years to learn more about what causes reading difficulties. The participants will have a 2-day testing of paper and pencil tasks, computerized tests, and Magnetic Resonance Imaging (MRI) scans of the brain. The tests, some scans, and school testing with summary are free. They are also interested in having unaffected siblings of children with NF-1 involved with the study, although their participation is NOT a requirement to be in the study. Travel reimbursement is provided for participating families.

"Mitochondrial Polymorphism and Number of Neurofibromas in NF-1"

Massachusetts General Hospital, Boston, Massachusetts
Andreas Kurtz, Ph.D. at (617) 726-5620
e-mail: kurtza@helix.mgh.harvard.edu

This study is to determine why some individuals with NF-1 develop a large number of neurofibromas (tumors) whereas others may develop a smaller number. This study explores the possibility of genes other than the NF-1 gene being involved in determining the number of tumors an individual may have.

"Molecular Genetic Basis of NF"

Massachusetts General Hospital, Boston, Massachusetts
Mia MacCollin, M.D. at (617) 726-5725
e-mail: maccollin@helix.mgh.harvard.edu

Fresh tumor specimens from patients with NF who are scheduled to have surgery in the near future are being collected to study the molecular genetic basis of NF. If you are scheduled for surgery and wish to participate, please contact Dr. MacCollin. This work has been approved by the Institutional Review Board of Massachusetts General Hospital.

"Cloning of the Schwannomatosis Locus"

Massachusetts General Hospital, Boston, Massachusetts

Mia MacCollin, M.D. at (617) 726-5725

e-mail: maccollin@helix.mgh.harvard.edu

They are seeking kindreds in which two or more individuals are affected with schwannomatosis (multiple schwannomas without tumors on the nerves to the ears). In addition, fresh tumor specimens from patients with schwannomatosis (with or without a family history) who are scheduled to have surgery in the near future are being collected to better understand the cellular biology of this condition. If you have questions about participation or eligibility, please contact Dr. MacCollin. This work has been approved by the Institutional Review Board of Massachusetts General Hospital.

"NF and Behavior Study"

North Shore/Long Island Jewish Research Institute, Washington, DC, Richmond, VA, Tulsa, OK

Gene Fisch, Ph.D. at (516) 240-8300.

e-mail: gfisch@nshs.edu

This study evaluates children with NF-1 between the ages of 5 and 13 years to determine the possible effects of genetic disease has on learning and behavior. The Stanford-Benet (4th edition) and the Vineland Adaptive Behavior Scale will be used. Testing takes about 1 ½ hours per child and the results will be shared with families at no cost. The study calls for the children to be re-evaluated in 2-year intervals.

"PEG-Intron Clinical Trial for Plexiform Neurofibromas"

Children's National Medical Center, Washington, DC

Roger Packer, M.D. at (202) 884-2652

e-mail: rpacker@cnmc.org

The Neurofibromatosis Program at Children's National Medical Center, Washington, DC, has started a clinical trial for the treatment of plexiform neurofibromas with PEG-Intron, a form of interferon. Individuals with plexiform neurofibromas younger than 21 years are eligible.

"Therapy of Children with Attention Deficit Disorder (ADHD) by Functional MRI"

Children's National Medical Center, Washington, DC

Roger Packer, M.D. at (202) 884-2652

e-mail: rpacker@cnmc.org

"NF-1 Tumor Growth Study"

University of Cincinnati, Cincinnati, Ohio

Nancy Ratner, Ph.D. at (513) 558-6079

e-mail: nancy.ratner@uc.edu

*This is an ongoing study of NF-1 tumor growth factors, growth factor receptors, and signaling pathways in neurofibroma cells. To enroll in this study, tumors must be removed by the patient's own physician who is to contact Dr. Ratner **24 hours BEFORE removal.***

"NF-1 Benign and NF-1 Malignant Tumor Study"

University of Florida, Gainesville, Florida

Peggy Wallace, Ph.D. at (352) 392-3055

e-mail: peggyw@flu.edu

Benign plexiform neurofibromas and neurofibrosarcomas (sometimes called MPNST) are being studied to understand the changes that cause growth of these tumors and to help develop better treatments. If you wish to participate in this study and as soon as a surgery date is set, please contact Dr. Wallace or Dr. Muir. One to three teaspoons of blood will be needed as well as the tumor tissue.

"Multi-Centered Study of Tibial Dysplasia in NF-1 Patients"

Shriners Hospitals for Children, Intermountain Hospital, Salt Lake City, Utah

Jeanne Siebert at (801) 536-3601

e-mail: jsiebert@shrinenet.org

John C. Carey, M.D. is conducting a study of NF1 and/or Tibial Dysplasia (TD) to determine the health status and health-related quality of life of children with NF1 for one year, the outcomes of adults with a diagnosis of Tibial Dysplasia at a point in time and the natural history of children with TD, with or without NF1 during a four year period of time. Participation in the study will involve clinic visits at participating facilities located mainly in the United States and Canada and telephone interviews at a time convenient for the participants. Obtaining various medical and outcome information will assist in determining what treatments and surgeries have the best results from a medical perspective and the participants' point of view.

"Phase II Study of Vinblastine and Methotrexate for NF-1 Progressive Plexiform Tumors"

Children's Hospital of Philadelphia, Philadelphia, Pennsylvania

Jean Bello Belasco, M.D. at (215) 590-3129

This is a clinical trial to determine the effect of vinblastine and methotrexate on time to disease progression in children or young adults up to 25 years of age who have progressive plexiform neurofibroma. Participating sites also include Spectrum Health, Grand Rapids, Michigan with Richard Axtell (616) 391-9366; Southwestern Medical Center, Dallas, Texas with principal investigator Gail Tomlinson (214) 456-8588.

"Phase II Trial of R115777 for NF-1"

National Cancer Institute, Bethesda, Maryland

Brigitte Widemann, M.D. at (301) 496-7387

e-mail: widemanb@mail.nih.gov

Frank Balis, M.D. at (301) 496-0085

e-mail: balisf@nih.gov

This is a clinical trial of an investigational drug called R115777 that blocks farnesyltransferase in patients 25 years and younger with NF-1 who have measurable growing plexiform neurofibromas.

"Phase II Trial of Pirfenidone for NF-1"

National Cancer Institute, Bethesda, Maryland

Brigitte Widemann, M.D. at (301) 496-7387

e-mail: widemanb@mail.nih.gov

Phase II clinical trial of pifafenidone, a drug being tested to control the growth of tumors, is open for children with NF-1 and plexiform neurofibromas between the ages of 5 and 18 years. Other participating sites are Children's National Medical Center, Washington, DC with Roger Packer, M.D. (202) 884-2652; Mayo Clinic, Rochester, Minnesota with principal investigator Dusica Babovic-Vuksanovic, M.D. (507) 284-8198.

"Proton Magnetic Resonance Spectroscopic Imaging in NF-1"

Johns Hopkins School of Medicine, Baltimore, Maryland

Peter B. Barker, D.Phil at (443) 923-9250

e-mail: pbarker2@jhmi.edu

Children with NF-1 between the ages of 6 and 16 who do not have any known illness are being studied to understand the effects of NF-1 has on the brain. Neuropsychological/psychoeducational testing and Magnetic Resonance Imaging (MRI) scans of the brain will be done at the Kennedy Krieger Institute in Baltimore. The testing takes about 2 hours, MRI takes about 1 hour (can be done the same day). Each participant receives \$50 and a free summary report of test results.

"NF2 Natural History Consortium Study"

House Ear Institute, Los Angeles, California

William H. Slattery III, M.D.; Shauna Duke at (213) 273-8008

e-mail: sduke@hei.org

The purpose of the study is to follow the natural history of all tumors related to NF2. Information from yearly MRI scans of the brain and spine, hearing tests, eye exams, neurological check-ups and physical functioning questionnaires will be collected for 3 years. To participate in the study, the NF2 diagnosis has to have been made on or after January 1, 1993. You must have at least one untreated acoustic neuroma, meningioma or spinal schwannoma which will remain untreated for the next 12 months.

Clinical Trials Sponsored by the National Institutes of Health

website: www.clinicaltrials.gov

Medical Journal Article Abstracts

website: www.nlm.nih.gov Click on PubMed

Neurofibromatosis, Inc., 9320 Annapolis Road, Lanham, Maryland 20706-3123

toll free: 1-800-942-6625 or e-mail: nfinfo@nfinc.org

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