

# THE WASHINGTON UNIVERSITY NEUROFIBROMATOSIS (NF) CENTER

*Making a Difference Together*

## A MESSAGE FROM THE DIRECTOR

### WELCOME!

The Washington University Neurofibromatosis (NF) Center is composed of clinicians and laboratory scientists focused on accelerating the pace of scientific discovery and its application to the care of individuals with NF.

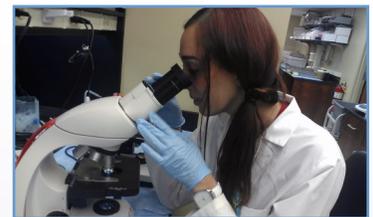
Our mission is to galvanize and promote research on NF, achieving significant breakthroughs in the diagnosis and treatment of this condition. We believe that these breakthroughs are possible when researchers, medical professionals, and families partner together.

The Washington University Neurofibromatosis (NF) Center comprehensive care team offers detailed patient evaluations and assessments. They work seamlessly with families, referring physicians, allied health professionals and other agencies to deliver the most advanced medical services available to children and adults affected by NF.



In 2014, we celebrate 10 years of excellence in the Washington University Neurofibromatosis (NF) Center as well as 20 years of providing multi-disciplinary care in the NF Clinical Program at St. Louis Children's Hospital. As director, I could not be prouder of our outstanding team of researchers and clinicians. Since its inception in 2004, we have grown to thirty members, published over 65 collaborative papers, and secured over 32 collaborative research grants. In this regard, the Washington University NF Center is one of the world's largest collaborative research enterprises focused on accelerating the pace of scientific discovery and its application to the care of individuals with NF.

To celebrate these milestones, we will be hosting our second Washington University NF Center Research Symposium on May 16, 2014 in the Eric P. Newman Education Center on the campus of the Washington University School of Medicine. We are also beginning several new initiatives designed to greatly enhance the research and clinical activities in the center. I invite you to follow our progress on the Washington University NF Center website.



Your partnership with the Washington University NF Center is essential to our mission that envisions a future where scientific advances inform better treatments for children and adults with NF. Thank you for your support.



**David H. Gutmann, MD PhD**

**Donaldo O. Schnuck Family Professor of Neurology**



Washington University  
Neurofibromatosis Center

Visit our website at: <http://nfcenter.wustl.edu>

## SAVE THE DATE!

CLUB NF  
FEBRUARY 1, 2014

Join us for art therapy as we create masterpieces for display at the 2014 Symposium

CLUB NF  
APRIL 5, 2014

Join us in pushing our limits at rock climbing!

RESEARCH SYMPOSIUM  
MAY 16, 2013

Join us at our bi-annual Research Symposium (details on final page)

## RESEARCH AT THE WASHINGTON UNIVERSITY NF CENTER

### WASHINGTON UNIVERSITY NF CENTER RESEARCHERS REPORT ON SLEEP DISTURBANCES IN CHILDREN WITH NF1

Amy Licis, MD and her colleagues in the Washington University NF Center recently completed a large study to better understand the sleep disturbances that affect children with NF1.

In this report, a cross-sectional study was performed on 129 children with NF1 and 89 of their unaffected siblings. She found that children with NF1 are more likely to have problems getting to sleep and staying asleep. In addition, parents of children with NF1 frequently report that their affected children have increased sweating at night and problems with getting up in the morning. However, children with NF1 do not have problems with abnormal sleep breathing or staying awake during the day.



Dr. Licis is currently planning on beginning new studies to more accurately measure sleep problems in children with NF1 as an initial step toward improving sleep and daytime school performance in these affected children.

Dr. Licis is an Assistant Professor in the Pediatric Sleep Center in the Department of Neurology at Washington University.

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### NEW CLUE TO AGGRESSIVE BRAIN TUMORS

*The following is a segment of an article that originally appeared in the Record on October 18, 2013. It was written by Michael C. Purdy.*



Scientists at Washington University School of Medicine in St. Louis have identified a biological marker that may help predict survival in people with deadly brain tumors. The researchers showed that when the marker is present at higher levels, brain cancers, known as glioblastomas, are more aggressive.

The cancer cells do not make the marker, a protein called F11R. Instead, it is made by non-cancerous cells, called monocytes, found within the tumor. Monocytes normally support and protect healthy brain cells, but they also can provide critical support to tumors.

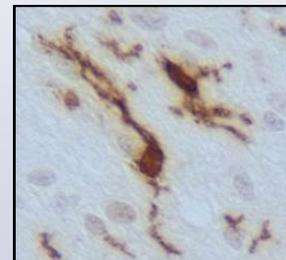
Glioblastomas are rare, but among the most dangerous tumors. Even with radiation and chemotherapy, the median survival rate is a little more than a year.

Hoping to provide another avenue of attack for these cancers, Gutmann and his collaborators have been studying how non-cancerous cells contribute to brain cancer formation and growth.

Winnie W. Pong, PhD, a staff scientist in Gutmann's laboratory, wanted to determine whether monocytes in the glioblastomas originate in the brain early in embryonic development or migrate into the brain from the bone marrow. Differences in where cells originate may affect their ability to support cancer development and growth.

To address this question, Gutmann turned to Elaine Mardis, PhD, co-director of The Genome Institute at Washington University, for help. Mardis has been a leader in developing techniques for sequencing RNA, the material cells use to copy protein-building instructions from DNA. The number of RNA copies of a gene present in a cell reflects how often the cell is using the gene to make protein.

F11R emerged as one of the best indicators of whether monocytes came from the brain or from bone marrow. F11R normally is made by monocytes that originate in the brain and not by those that come from bone marrow.



To find new treatments for these deadly cancers, Gutmann and his colleagues are currently working to identify factors made by monocytes that help the tumors grow.

## MAKING A DIFFERENCE TODAY!

### COURTNEY'S CORNER: INSPIRED TREE HOUSE ACTIVITIES PROMOTE DEVELOPMENT AND WELLNESS IN CHILDREN

Often our families come to me with general concerns about their child's development. Many of our children experience some developmental delays and sometimes those delays occur within motor skill development. Children with NF1 can experience trouble with skills such as balancing, motor planning, grasping objects and endurance.

What families may not realize is that there are so many easy ways to help children work on these skills right at home! My friend and colleague Pam Braley OTR/L, along with other specialists in physical and occupational therapy, has started a blog called *The Inspired Tree House* (<http://theinspiredtreehouse.com/>), which suggests everyday activities to promote development and wellness in children.



What do I love about this blog? It is a fantastic introduction on how to develop skills in children, and you do not need a degree in physical or occupational therapy to understand it. Start by checking out the "Clinical Close-Up" tab. This page will give you a quick overview of all the language physical therapists and occupational therapists use to describe different motor skills empowering you to understand exactly which skills your child needs help with.



Once you feel like you've gotten a good handle on the basic terminology, explore the "Categories" section. Each category is a specific motor skill and clicking on one will take you directly to activities that will help develop that particular skill in your child. Not only will you be given step-by-step instructions, but also ideas for ways to change it up and make it more challenging as your child reaches new levels of ability.

Helping your children achieve new levels of success takes work, but it is such a rewarding experience for parents and children alike. Playing together and trying new things is an easy way to get started. Go get inspired!

**Courtney Dunn, PT DPT** (pictured in action above) is a Washington University NF Center Physical Therapist.

### WASHINGTON UNIVERSITY NF CENTER FAMILY SHARES FUNFEST SUCCESS



On November 11, 2013, Brain and Amanda Walk and their daughters visited the Washington University NF Center to celebrate another successful year of fuNFest—the annual festival that supports research at the Washington University NF Center. Despite the rain, fuNFest was tremendous fun, including games, food, friends and activities coordinated by Club NF team members.

The Washington University NF Center extends its heartfelt gratitude to Amanda and Brain Walk, who worked tirelessly to plan this event. We consider our families to be an integral member of Team NF. Because of our families, we are able to conduct cutting-edge research and to provide outstanding resources, like Club NF.



## WASHINGTON UNIVERSITY NF CENTER EVENTS

### SECOND WASHINGTON UNIVERSITY NF CENTER RESEARCH SYMPOSIUM

Please join us on May 16, 2014 for the second Washington University NF Center Research Symposium.

Our keynote speakers are:

**Dr. Sean Morrison**, Mary McDermott Cook Chair in Pediatric Genetics  
Director, Children's Medical Center Research Institute at the University of Texas—Southwestern  
Investigator, Howard Hughes Medical Institute

**Dr. Johnathan A. Epstein**, William Wikoff Smith Professor of Medicine  
Chair, Department of Cell and Developmental Biology  
Scientific Director, Penn Cardiovascular Institute at the Perelman School of Medicine at the University of Pennsylvania

In addition, there will be presentations on multiple aspects of NF1 ranging from a molecular understanding of specific NF1-associated features to how to provide meaningful care to individuals with NF1. We anticipate that this will be an engaging and informative experience. We encourage all to attend!

For details, please contact Kirsten Brouillet at [brouilletk@neuro.wustl.edu](mailto:brouilletk@neuro.wustl.edu)

### SAVE THE DATE



**MAY 16, 2014 | 8AM-4PM**

**Eric P. Newman Education Center  
Seminar Room B**

### ANNOUNCING THE 2014 CLUB NF SCHEDULE!



**February 1, 2014**  
Club NF Creates Art!



**April 5, 2014**  
Club NF Rocks!



**June 7, 2014**  
Club NF Zoo Hunt!



**August 2, 2014**  
Club NF Swims!



**October 4, 2014**  
Club NF Goes to fuNFest!



**December 6, 2014**  
Club NF Making Music!

### STAY CONNECTED!

#### WEBSITE



#### FACEBOOK



#### YOUTUBE

