

# WASHINGTON UNIVERSITY NEUROFIBROMATOSIS CENTER

EXCEPTIONAL CARE *through* GROUNDBREAKING RESEARCH

## WALK FAMILY MAKES DONATION TO SUPPORT NF RESEARCH

On November 11, the Walk family presented a check for the full amount of the proceeds from their annual fuNFest fundraiser. The Washington University NF Center thanks them for their continued support of NF research!

“The Walk family’s support is a driving force in our effort to make personalized NF medicine a reality. Their passion is my inspiration, and I am so thankful for their hard work and devotion to furthering NF research.”

- Corina Anastasaki, PhD, Gutmann Research Laboratory



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## UNDERSTANDING THE DIVERSE CLINICAL FEATURES OF NF1

Individuals with Neurofibromatosis type 1 (NF1) are more likely to develop benign and malignant tumors of the nervous system. In addition, they are prone to numerous other medical problems, ranging from bone defects to seizures. Because of this heterogeneity, it is critical that treating physicians are aware of the diverse manifestations of this disorder.

A recent review of NF1, published by Angie Hirbe, MD, PhD, a post-doctoral research fellow in the laboratory of Dr. David Gutmann at the Washington University NF Center, offers a detailed look at the clinical features and various tumors which frequently affect individuals with NF1.

Typically the earliest symptoms of NF1 are café-au-lait macules, followed by skinfold freckling and Lisch nodules, harmless pigmented lesions of the eye. Many individuals will also develop neurofibromas, skeletal deformities, neurocognitive deficits and cardiovascular

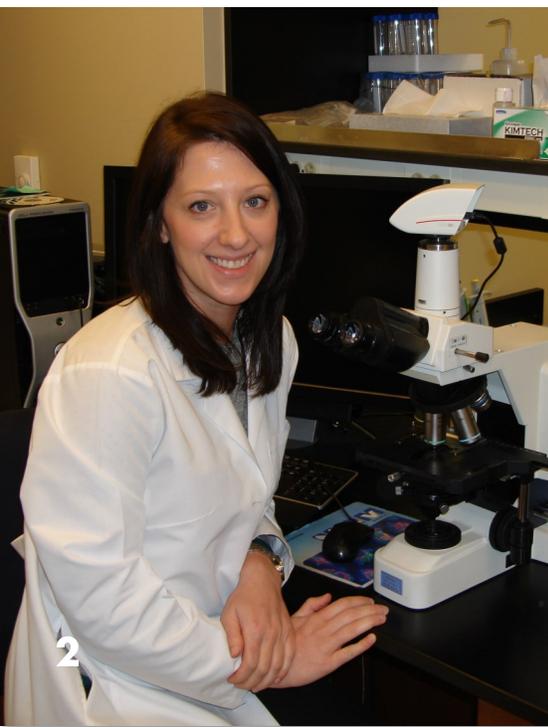


“ Hirbe advocates a multidisciplinary approach to care ...to ensure optimal management of people with NF1. ”

abnormalities to varying degrees. Beyond the non-malignant clinical features, Hirbe reports a 15-20% incidence of low-grade central nervous system tumors, with roughly 80% occurring in the optic pathway. Individuals with NF1 also have an increased risk of developing other types of tumors, including glioblastomas (malignant

brain tumors), nerve tumors (malignant peripheral nerve sheath tumors), breast cancers, leukemia and lymphoma.

For this reason, Hirbe advocates a multidisciplinary approach to care, entailing a team of specialists, to ensure optimal management of people with NF1.



## RESEARCHER PROFILE:

SIDNEY WILLIAMS, PhD

Sidney is our newest member of the Washington University NF Center Research Laboratories. She earned her bachelor's degree in Neuroscience in 2007 at Furman University, and received her master's degree in Behavioral Neuroscience in 2009 at Florida Atlantic University. She went on to complete her PhD in Neurobiology in 2014 at the University of Alabama, Birmingham.

Her current research focuses on how the *NF1* gene controls nerve cell function in the part of the mouse brain responsible for learning and memory.

## NEW DRUG SHOWS PROMISE FOR NF1 PLEXIFORM NEUROFIBROMAS



**Dr. Brian Weiss, MD**

Weiss, a pediatric oncologist at Cincinnati Children's Hospital, specializes in the research and treatment of neuroblastoma, a

Plexiform neurofibromas are common benign nerve tumors seen in children and adults with neurofibromatosis type 1 (NF1). While these tumors are typically slow growing, some may require treatment.

In a study spearheaded by Dr. Brian Weiss at Cincinnati Children's Hospital and conducted as part of the Department of Defense-funded NF Clinical Trials Consortium, Sirolimus was evaluated as a potential chemotherapy agent for plexiform neurofibromas.

Sirolimus is a drug similar to rapamycin, a compound first shown

to be effective for reducing the growth of mouse *Nf1* optic gliomas in the laboratory of Dr. David Gutmann of the Washington University NF Center.

Dr. Weiss and colleagues found that Sirolimus decreased the time it took for a tumor to grow ("time to progression") by almost four months. While this result is modest, it does suggest that some plexiform neurofibromas may respond favorably to this treatment.

Future work using related drugs as well as other classes of chemotherapy agents are currently being evaluated.

## FALL 2014 BEAT NF PROGRAM ANOTHER SUCCESS

We recently had our last session of the fall 2014 Beat NF program, and I already know I will miss my five little friends next Wednesday morning. Beat NF, a jazz, music therapy program for toddlers with NF1, concluded this morning after six successful weeks. Through the collaborative efforts of Jazz St. Louis, the Washington University Neurofibromatosis (NF) Center, and St. Louis Children's Hospital (SLCH), a unique play-based therapy group for toddlers was created. I have worked side by side with our SLCH therapist, Courtney Dunn, PT, DPT, and musicians from Jazz St. Louis, and I am amazed at the way we have all evolved both individually and as a group.

During each class, our goal is to assess and help improve the motor

skills, balance and coordination of each child. Another goal has been to work on social skills; look people in the eye when you shake their hand or introduce your friend with a clear, confident voice. Some of these skills were easy from week one or two, while others took weeks to develop. Because each week builds on the last (with four repeated songs that have new motor challenges), the children could grow in confidence as they mastered a new skill or learned the words to a song. What a success for all involved!

You might be wondering how motor therapy and jazz music are connected. Jazz music has evolved over many decades citing many different influences, such as blues and ragtime. It involves a complex blend of syncopation (different

rhythms working together) and improvisation (creating music without preparation). While we carefully planned our weekly motor goals, jazz music allowed each child to improvise, create and explore in a small, safe setting.

>>>Read more about the fall 2014 Beat NF program at <http://nfcenter.wustl.edu/fall-2014-beat-nf-program-another-success/>





## UPCOMING EVENTS

MARK YOUR CALENDAR!

**FEBRUARY 7, 2015**

Club NF Surfs  
at Core 3  
(indoor surf studio)

**APRIL 4, 2015**

Club NF Play-Based Therapy  
Event Details  
COMING SOON!

Donations to the Washington University Neurofibromatosis (NF) Center enable our mission to find better treatments for children and adults with neurofibromatosis (NF).

**Washington University is a federal tax-exempt organization.**

**1** Visit [gifts.wustl.edu](http://gifts.wustl.edu), select "NF Center" in the Centers and Institutes drop-down menu and complete the form to submit a monetary donation to support research at the Washington University NF Center. Financial contributions may be provided as an endowment, bequest, gift, or tribute to a loved one with neurofibromatosis.

**2** Register to receive a FREE Schnucks eScrip Community Card, have it scanned each time you shop at Schnucks Markets and Schnucks will donate a percentage of your grocery bill to the Washington University NF Center.

Just by shopping at Schnucks and scanning your community card, you are supporting the Washington University NF Center!

Learn more about the program at [nfcenter.wustl.edu/giving/shopfornf](http://nfcenter.wustl.edu/giving/shopfornf).

**3** The Washington University NF Center has partnered with Bravelets to help families and individuals affected by NF. With each Bravelets item purchased, \$10 is donated to the Washington University NF Center.

There are a variety of Bravelets items to choose from including bracelets, necklaces and rings for both men and women. They make excellent holiday and birthday gifts, and serve as a reminder to be brave throughout all of life's challenges.

Shop for Bravelets at [bravelets.com](http://bravelets.com).

## THREE GREAT WAYS TO SUPPORT THE WASHINGTON UNIVERSITY NF CENTER

**STAY CONNECTED >>** [nfcenter.wustl.edu](http://nfcenter.wustl.edu)

