

THE WASHINGTON UNIVERSITY NEUROFIBROMATOSIS (NF) CENTER

Exceptional Care through Groundbreaking Research

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.

Our comprehensive care team offers detailed patient evaluations and assessments. We work seamlessly with families, referring physicians and allied health professionals to deliver the most advanced medical services available to children and adults affected by NF.



Washington University
Neurofibromatosis Center

WASHINGTON UNIVERSITY NF CENTER FAMILY CELEBRATES FINAL DAY OF CHEMOTHERAPY



Children with NF1 are prone to develop a type of brain tumor that affects the nerve which carries vision from the eyes to the brain, known as an optic pathway glioma (OPG). In some instances, these tumors cause progressive vision loss, and require treatment with chemotherapy. Ally is one of our children with NF1 and a progressive OPG. A six-year-old girl now preparing for first grade,

Ally has been battling her OPG since August 2011. After four different types of chemotherapy and one surgery, we are happy to report that Ally's tumor is stable.

On August 4, 2014, Ally received her final dose of chemotherapy. As is the tradition for children cared for by the Pediatric Neuro-Oncology team, Ally celebrated her big day by ringing the bell in the Oncology wing at St. Louis Children's Hospital. Friends, family, physicians, nurses, and Team NF were there to cheer her on. Even Ronald McDonald made a special appearance.

"It was a beautiful moment," said Team NF Coordinator Kirsten Brouillet, "Seeing the family together, celebrating and smiling, really puts everything into perspective."

Please join us in congratulating Ally and her family on this momentous occasion.



SAVE THE DATE!

**CLUB NF— fuNFest
OCTOBER 4, 2014**

Join us at the annual NF fundraiser—fuNFest.

**BEAT NF
OCTOBER 14, 2014**

Enjoy six Tuesday morning music therapy sessions.

**CLUB NF—
DECEMBER 6, 2014**

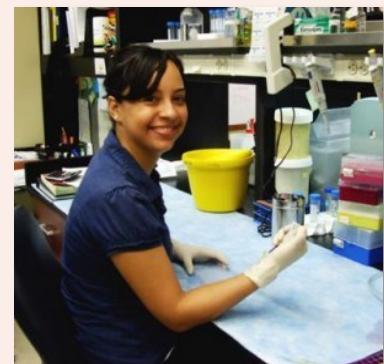
Join us at this fun play-based therapy event!

RESEARCH AT THE WASHINGTON UNIVERSITY NF CENTER

RESEARCHERS DEFINE POTENTIAL TARGET FOR NF2-ASSOCIATED SPINAL TUMOR TREATMENT

Individuals with NF2 are at increased risk for developing spinal cord tumors called ependymomas. Currently, the only available treatment for these tumors is surgery.

Cindy Garcia, PhD, a former graduate student in the laboratory of David H. Gutmann, MD, PhD, used genetically-engineered mouse models of NF2 to determine how the *Nf2* protein, merlin, controls spinal cord cell growth. She found that merlin suppresses the activity of a critical growth factor receptor, called ErbB2, and that blocking ErbB2 activation in NF2-deficient cells inhibited their growth. These findings offer potential targets for new drug treatments.

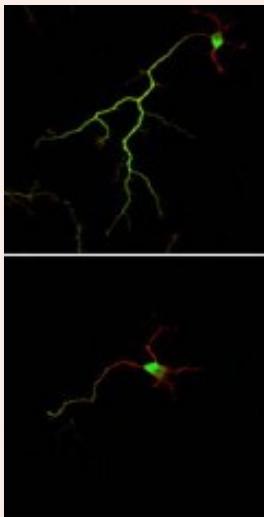


RESEARCHERS PUBLISH FINDINGS ON NEW PLEXIFORM NEUROFIBROMA TREATMENT

Plexiform neurofibromas, tumors that grow from nerves throughout the body, are more prevalent in individuals with NF1. These tumors are among the most debilitating features of NF1; however, there are currently no standard treatment options for reducing the size of these tumors.

Brian Weiss, MD, of Cincinnati Children's Hospital and his collaborators, including David H. Gutmann, MD, PhD, found that a new treatment called sirolimus, while effective in keeping tumor size stable, does not cause reduction of tumor size. These findings indicate the continued need for NF1 clinical trials focused on finding effective treatments for the reduction of plexiform neurofibromas.

NEW ANALYSIS PROVIDES ADDITIONAL SUPPORT FOR FINDINGS ABOUT GENDER AND OPTIC PATHWAY GLIOMA



Kelly Diggs-Andrews, PhD, and her colleagues recently reported that females with NF1 and an optic pathway glioma are at higher risk for vision loss than are males. The study by Dr. Diggs-Andrews, a former post-doctoral fellow in the laboratory of David H. Gutmann, MD, PhD at the Washington University NF Center, was published earlier this year in the *Annals of Neurology*.

Michael J. Fisher, MD, at the Children's Hospital of Philadelphia, took specific interest in these findings, and decided to reanalyze data that he previously reported on children with NF1 and optic pathway gliomas. In agreement with Dr. Diggs-Andrews' study, he likewise found that females with NF1 and an optic nerve glioma were five times more likely to have vision loss than boys.

These findings led Dr. Gutmann and his team to take a second look at their own data. When limited to children with optic nerve tumors only, females were 10 times more likely to develop vision loss and require treatment than their male counterparts.

These findings underscore the need to further study gender as a risk factor in NF1.

MAKING A DIFFERENCE TODAY!

BEAT NF: UNDERSTANDING HOW MUSIC THERAPY HELPS CHILDREN WITH NF1

Guest Blog from Maryville Music Therapy Department Director Cynthia Briggs, Psy.D.



The components of music include rhythm, tonality, meter, phrasing, accent, melody, harmony and lyrics. In music therapy, these components become the tools used to address specific therapeutic needs and to facilitate skill development. As a music therapist, my goal is to understand what types of skills a person in therapy is seeking to attain, and then find a way to integrate the potential for skill development into music. During Beat NF, we look at multiple skills that are commonly delayed in children with NF1 and find ways to integrate those skills directly into jazz music.

Learning and Attention

Many children with NF1 experience challenges with learning and attention as well as processing and retrieving information. Because the structure of music organizes information and facilitates memory and retrieval, it is the perfect tool for developing learning skills. One of our many goals during Beat NF is to create songs that require children to follow specific steps and actions in order to learn a new skill. By breaking skills down into simple steps, children have the opportunity to better process and retrieve new information.

Executive Function

Executive function, or the ability to plan, problem-solve and execute solutions, is also an area that is often delayed in children with NF1. Music relies on components such as rhythm and meter in order to exist. These components readily lend themselves to sequencing activities. A child learns that a pause in the music means you wait and, in order to stay on the beat with your peers, you must attend to your surroundings. At Beat NF, children have the opportunity to play a special instrument called an Orff Instrument, similar to a xylophone. We challenge children to play on a beat rather than simply playing randomly. By practicing this skill, children work on planning and managing impulsive behaviors.

Language Skills

Children with NF1 also experience delays in expressive (spoken) and receptive (heard) speech. Because music is built from the same elements as speech (rhythm, tonality, inflection, accent), it is an excellent way to practice language skills. When words are set to music, it can help children to attain more fluid speech. Encouraging children to sing a phrase and then speak the same phrase can help them transfer the fluidity of music into their spoken language.

Motor Skills

Motor skills, both fine or finger-based and gross or whole body-based, can be delayed in children with NF1. Music can be used to encourage movement in multiple ways. First, it can be written directly into a song. At each Beat NF session, we play an original Motor Song which encourages children with NF1 to practice specific motor skills such as jumping and balancing. Even without writing the specific movements into a song, music can still be used to encourage motor development. Setting music as a background to movement can encourage fluidity. Changing the meter of the music from slow to fast can encourage children to change the rate of their movements. There are endless ways to encourage motor development through music.



JOIN US AT THE UPCOMING FALL 2014 BEAT NF!

WHEN: Wednesday mornings, October 15–November 19, 2014, 9:30–10:45 AM

WHERE: The Harold and Dorothy Steward Center for Jazz
3536 Washington Ave., St. Louis, MO 63103

GET INVOLVED!



JOIN US AT fuNFest 2014!

Join team NF at the 4th annual fuNFest at Gatch Lake (near Vandalia, IL) on Saturday, October 4 from 12 p.m. to 6 p.m. The Walk family sponsors this annual fundraising event and generously donates all proceeds to St. Louis Children's Hospital Foundation/Washington University NF Center. Attendees can participate in fun games, play in bounce houses, get your face painted and play the infamous Cow Patty Bingo for a chance to win \$1,000! Food and drinks are available for purchase.

To help support this exciting event, Club NF will be in attendance and Team NF therapists will lead a variety of games and activities which reinforce core strength and gross motor skills.

If you are planning to attend Club NF and are interested in riding a free charter bus from the Washington University Danforth campus (leaving campus at 9:30 a.m. and returning to campus around 5:30 p.m.), please contact Kirsten Brouillet at brouilletk@neuro.wustl.edu no later than September 29.

STAY CONNECTED!



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