

# NF Family Day 2020

Thank you to the Children's Tumor Foundation and the Gilbert Family Neurofibromatosis Institute for sponsoring NF Family Day 2020!



# MULTIDISCIPLINARY CLINIC

The Multidisciplinary Neurofibromatosis team at Children's National Hospital offers comprehensive diagnostic evaluations and follow-up care for children and adolescents with known or suspected NF and their families. Our team includes specialists from many different medical areas, allowing us to provide the best possible medical care and psychosocial supports to our patients and families!

## Meet Our Team!

- Dr. Miriam Bornhorst, Clinic Director, Neuro-Oncologist
- Dr. Ryan Uy, Developmental and Behavioral Pediatrician
- Dr. Laura Kurzius, Psychologist
- Dr. Karin Walsh, Neuropsychologist
- Dr. Kristina Hardy, Neuropsychologist
- Peter Shibuya, Nurse Practitioner
- Jennifer Ramos-Blanco, Clinic Coordinator



# EXPERIENCE AT CHILDREN'S NATIONAL

## Description:

The NF research team at Children's National does research with medical data from patients who have NF1 and other RAS/MAPK Pathway related syndromes. The goal of this research is to determine the number of children with NF1 and other RAS/MAPK Pathway related syndromes seen at the Children's National and to determine the incidence of associated clinical problems.

## In order to participate, you must:

- Have been diagnosed with NF1 or another RAS/MAPK Pathway related syndrome and receive care at Children's National Hospital

## Who to contact?

- Hannah Weisman, Study Coordinator: [hweisman@childrensnational.org](mailto:hweisman@childrensnational.org)
- Dr. Miriam Bornhorst, Principal Investigator: [mbornhorst@childrensnational.org](mailto:mbornhorst@childrensnational.org)



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# NF1 FRAMESHIFT PEPTIDES EXPLORATORY STUDY

## Description:

This is a research study to find out if children and young adults with NF1, and either Low-Grade Gliomas (LGGs) or Plexiform Neurofibromas (PNs), have a specific frameshift peptide protein profile. The term frameshift refers to genetic changes caused by insertions or removal of proteins that make up the gene in a DNA sequence. Peptides are short chains of amino acids linked by peptide bonds. We want to study these mutations and determine whether a disease specific vaccine can be created to address these mutations.

## Who to contact?

- Bergen Kassoff, Study Coordinator: [bkassoff@childresnational.org](mailto:bkassoff@childresnational.org)



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# RELIABILITY AND VALIDITY OF COMPUTERIZED COGNITIVE OUTCOME TOOLS IN NF1

## Description:

The purpose of this study is to see if two newer computer-based cognitive test batteries are good at assessing learning in children with NF1. It is important for the tests we use to measure learning to 1) test the areas of learning that are most important to children with NF1 and 2) be dependable in measuring performance over time. We think that children with NF1 may not show the same consistency over time on these tests. This is important for us to know so that we can choose the best tests or make the tests better for future studies in NF1.

## Who to contact?

- Hannah Weisman, Study Coordinator: [hweisman@childrensnational.org](mailto:hweisman@childrensnational.org)
- Dr. Karin Walsh, Principal Investigator: [kwash@childrensnational.org](mailto:kwash@childrensnational.org)



# EXAMINING THE RELATION BETWEEN LEARNING AND ATTENTION DEFICITS AND SLEEP DISTURBANCE IN CHILDREN WITH NF<sub>1</sub>

## Description:

Concerns regarding sleep and difficulties with attention and learning are common in children with NF<sub>1</sub>. The goals of this study are to 1) understand how common sleep disturbance is and what kinds of sleep problems children with NF<sub>1</sub> have and 2) look at the relationship between sleep disturbance and attention and learning difficulties in children with NF<sub>1</sub>.

## Who to contact?

- Hannah Weisman, Study Coordinator: [hweisman@childrensnational.org](mailto:hweisman@childrensnational.org)
- Dr. Karin Walsh, Principal Investigator: [kwalsh@childrensnational.org](mailto:kwalsh@childrensnational.org)



# MULTIMODAL INTERVENTION TRIAL FOR COGNITIVE DEFICITS IN NF1: EFFICACY OF COMPUTERIZED COGNITIVE TRAINING AND STIMULANT MEDICATION

## Description:

Children with NF1 are more likely to have problems remembering things and paying attention. A team of doctors at Children's National is testing a computer program that might help improve memory and attention in children with NF1. This program has been helpful for a small sample of children with NF1, but we now want to know if this program will help children with NF1 on a larger scale.

## In order to participate, you must:

- Have an NF1 Diagnosis based on National Institute of Health (NIH) criteria
- Be between ages 8 and 16 years old
- Speak English fluently and have a caregiver who speaks English fluently
- Not take any medicine for ADHD or if your child is taking an ADHD medication, the dose must have not changed for 30 days before joining the study

## Who to contact?

- Hannah Weisman, Study Coordinator: [hweisman@childrensnational.org](mailto:hweisman@childrensnational.org)
- Dr. Kristina Hardy, Principal Investigator: [kkhardy@childrensnational.org](mailto:kkhardy@childrensnational.org)



# PHASE 2 STUDY OF CABOZANTINIB IN CHILDREN (<16) WITH PLEXIFORM NEUROFIBROMAS

## Description:

This is a study of an oral tyrosine kinase inhibitor (taken once daily continuously) to see how well this can treat plexiform neurofibromas in children who have symptoms associated with their tumors.

## Who to contact?

- Dr. Miriam Bornhorst, Principal Investigator: [mbornhorst@childrensnational.org](mailto:mbornhorst@childrensnational.org)



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# MIRDAMETINIB (PD0325901) IN CHILDREN AND ADULTS WITH PLEXIFORM NEUROFIBROMAS

## Description:

This is a study of an oral Mek-inhibitor (twice daily; 3 weeks on, 1 week off) to see how well this can treat plexiform neurofibromas in children and adults who have symptoms associated with their tumors.

\*Pre treatment biopsy is required for adults who enroll on this study

\*Only tablet form available now, but liquid formulation will be available in 4-6 months

## Who to contact?

- Dr. Miriam Bornhorst, Principal Investigator: [mbornhorst@childrensnational.org](mailto:mbornhorst@childrensnational.org)



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# COG STUDY OF SELUMETINIB (AZD6244) VS CARBOPLATIN/VINCRIStINE FOR NEWLY DIAGNOSED OPTIC PATHWAY GLIOMAS

## Description:

This study is for children with NF1 who are newly diagnosed with an optic pathway glioma and need treatment. Children will be randomized to either receive the “gold standard” treatment (Carboplatin/Vincristine) or an oral Mek-inhibitor (Selumetinib) for treatment  
\*Mek-inhibitor is capsule only (no liquid)

## Who to contact?

- Dr. Miriam Bornhorst, Principal Investigator: [mbornhorst@childrensnational.org](mailto:mbornhorst@childrensnational.org)



# MEK<sub>1/2</sub> (BINIMETINIB) FOR THE TREATMENT OF PROGRESSIVE OPTIC PATHWAY GLIOMAS IN CHILDREN WITH NF<sub>1</sub>

## Description:

This is a study of an oral Mek-inhibitor for children with NF<sub>1</sub> who have a progressive optic pathway glioma (must have had growth after receiving treatment with something else) and need treatment.

\*\*Mek-inhibitor can be either a liquid or capsule

## Who to contact?

- Dr. Miriam Bornhorst, Principal Investigator: [mbornhorst@childrensnational.org](mailto:mbornhorst@childrensnational.org)



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The Children's Tumor Foundation's mission is to drive research, expand knowledge, and advance care for the NF community!

Find resources and events at: <https://www.ctf.org/>



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# EXPAND KNOWLEDGE



## 2020 NF1 Forum

The 2020 NF1 Family Forum series will be held in collaboration with NYU Langone and provide four 60-minute webinar sessions:

*6/25 – Poster Session*

*7/16 – Case Conference*

*8/13 – Clinical Research Updates*

*9/17 – Relationships, Learning, and School*

## 2020 NF2 Forum

October 2020 – pending final decision (coronavirus) - partnering with the Ohio State University, Columbus, OH

# race FOR every child

SAVE THE DATE

OCTOBER 17, 2020



Held in the heart of the nation's capital, the Race for Every Child is perfect for avid runners, weekend joggers, parents looking for a fun activity for their family, or anyone who wants to help ensure that every child can receive exceptional care. Join our NF team and help our kids grow up stronger!

**Where:** Freedom Plaza, Washington, DC

**Event Website:**

[https://giving.childrensnational.org/site/TR;jsessionid=00000000.app252a?fr\\_id=1240&pg=entry](https://giving.childrensnational.org/site/TR;jsessionid=00000000.app252a?fr_id=1240&pg=entry)



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# SHINE A LIGHT NF WALK

The Shine a Light NF Walk brings NF out of the shadows, raising awareness and funding research to help better the lives of those living with NF. Join us and come together with your community to Shine a Light on NF!

Virtual via Zoom or Facebook Live

<https://www.ctf.org/events/shine-a-light-nf-walk-washington-dc>

<https://www.ctf.org/events/shine-a-light-nf-walk-virginia>

October 3, 2020, Washington DC

September 12, 2020 Richmond, VA



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## NF Camp - Virtual program

July 18-24 - NF Heroes ages 18-25

July 25-31 - NF Heroes ages 12-17

Brainy Camps is officially transitioning to virtual camp for this summer! Each day will begin and end with a Zoom session for all campers and then multiple smaller breakout sessions will be offered throughout the day where campers can choose activities that are of interest to them (i.e., crafts, yoga, scavenger hunt, games, etc.). They also will have the opportunity to participate in support groups and educational sessions as well as meet together in “bunks” with their assigned counselors and bunkmates to create a feeling of unity and connection. In the evening, the campers will participate in camp-wide virtual group activities like a “campfire” sing-along and storytelling, talent show, and dance party.

Registration: <https://www.ctf.org/understanding-nf/nf-camp>





# RESOURCES

**Please feel free to reach out to us!**

Carly Berger: [cberger@childrensnational.org](mailto:cberger@childrensnational.org)

Danielle Griffin: [dgriffin2@childrensnational.org](mailto:dgriffin2@childrensnational.org)

Hannah Weisman: [hweisman@childrensnational.org](mailto:hweisman@childrensnational.org)

## Links:

- [ctf.org](http://ctf.org)
- You will have access to view this presentation on 6/20/20 via our Children's National site: <https://childrensnational.org/departments/center-for-neuroscience-and-behavioral-medicine/programs-and-services/the-gilbert-family-neurofibromatosis-institute>