

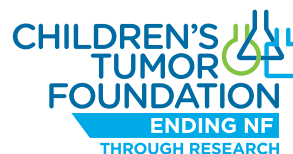


— 3<sup>RD</sup> ANNUAL —

# NEUROFIBROMATOSIS

## Young Investigators' Forum

An Academic Research Forum Focusing on Research Productivity, Career Development, and Community Building for Junior Faculty, Fellows, and Postdoctoral Researchers Conducting Impactful Work in Neurofibromatosis





# Message from the Activity Chairs

Dear Colleagues,

As the mechanisms underlying the various manifestations of neurofibromatosis type 1 (NF1) gradually come to light, new opportunities are emerging to detect complications earlier, treat them, and eventually even prevent them. The hope of achieving these goals is attracting an expanding group of young clinicians and scientists who will carry forward the critical research and efforts at clinical implementation. Their insights will help to open doors none of us could have imagined or predicted. This hope for the future was nowhere more evident than in the third Neurofibromatosis Young Investigators Forum (NFYIF), held in Baltimore, Maryland, on November 30, 2023. Following a competitive process, 19 graduate students, postdoctoral fellows, and junior faculty gathered to describe their research before a panel of Expert Judge Mentors with decades of experience in working on neurofibromatosis. Aside from attending the research presentations by the young investigators, the Mentors offered sage advice to participants on topics that ranged from perspectives on their research to how to position themselves for a successful career in neurofibromatosis research. Participants also had the opportunity to hear from and interact with a patient ambassador, who provided insights on her personal journey with NF1 and conveyed the sense of urgency to find solutions for this debilitating disorder.

We congratulate all the young investigators who took part in the 2023 NFYIF, both for the quality of their research and for their commitment to helping to end the burden of NF1 for patients and their families. It is events like this that justify optimism for continued advances on behalf of all those who live with NF1 every day.

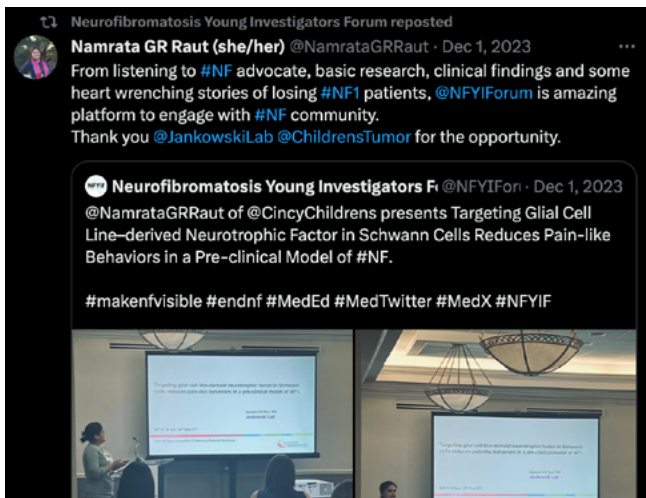
Sincerely,

*Bruce R. Korf, MD, PhD (chair)*

*Andrea M. Gross, MD (co-chair)*



2023 NFYIF Faculty & Young Investigators



“Participating in the NFYIF as a PhD candidate was a valuable chance to connect and receive guidance from experienced young investigators and established faculties in the NF field.



Thanks to the intervention of an NF patient representative, the forum emphasized the urgency of finding cures for all NF patients. As a scientist working primarily in a lab setting, this was exceptionally motivating and stimulating.”



“This was an amazing and inspirational opportunity!”



## About the NFYIF

The Neurofibromatosis Young Investigators' Forum (NFYIF) is a unique educational opportunity designed for young investigators (MD, DO, and/or PhD)—based in the United States and internationally—who are pursuing a career in academic research focused on neurofibromatosis across basic, translational, and clinical research settings. As a competitive academic research program, the NFYIF provides a professional venue at which oncology junior faculty and fellows are invited to submit an abstract of their unpublished, original research to a panel of expert faculty for assessment.

The NFYIF has been designed to ensure a high level of science, quality, and participation as a means of laying a substantive and healthy foundation for future years to build upon. CEC Oncology composed and conducted an international Call for Abstracts (CFA) among clinician scientists, research scientists, clinical fellows, and postdoctoral fellows involved in neurofibromatosis research across the full spectrum of disease (NF1, NF2, and schwannomatosis). After a rigorous, blinded selection process as determined by top scientific experts and thought leaders in the field, a highly select group of 19 researchers were invited to present their data to peers and an esteemed panel of Expert Judge Mentors in a modified National Institutes of Health (NIH) scoring format. In an effort to augment the professional development aspects of this forum, professional presentation skills coaches with a long history working with scientists to improve their ability to clearly present complex data, effectively and articulately address challenging questions, and manage tight time windows with professionalism and finesse were made available to all young investigators. The overarching goal of this initiative was and is to help identify, cultivate, and prepare young investigators for successful careers that help advance the field of neurofibromatosis via a “connect the unconnected” approach focused on collaboration, collegiality, and community-building, which are all especially crucial in the research niche of a rare disease like NF. Our third annual NFYIF installment robustly achieved this goal, and in so doing, effectively laid the groundwork for future successes.



“Recently, I had the pleasure of speaking at the Young Investigators Forum and was blown away by the passion each of the young investigators had for their research to help find a cure for neurofibromatosis. As a patient advocate and the 2023 Children's Tumor Foundation Ambassador, I know firsthand the importance of working together collectively to find a cure. Witnessing the camaraderie and team effort provided at this forum was truly inspiring.”



—Michele Holbrook, NF Survivor and Patient Advocate  
2023 National Ambassador for the Children's Tumor Foundation (CTF)



# The Neurofibromatosis Distinguished Young Investigator Award

The Neurofibromatosis Distinguished Young Investigator Research Award provides recognition for junior faculty who continue their dedication and sustained research efforts within neurofibromatosis. This year's Neurofibromatosis Distinguished Young Investigator Award was presented to:



## Stephanie Brosius, MD, PhD

*Instructor in Pediatrics*

*University of Pennsylvania, Children's Hospital of Philadelphia  
Philadelphia, Pennsylvania*

### Interneurons That BiTE: A Novel Cellular Therapy for High-grade Glioma

Stephanie Brosius, MD, PhD, is a pediatric neuro-oncologist and physician-scientist trained in molecular neuroscience who seeks to comprehend the contribution of the tumor microenvironment in the development and treatment of pediatric brain tumors, particularly those associated with NF1. Her overarching goal is to devise novel cellular therapies with a current focus on the use of migratory cortical interneuron precursors as a therapeutic delivery system. Dr. Brosius completed her medical degree and doctorate training in the Medical Scientist Training Program at the University of Alabama–Birmingham where she studied the Schwann cell mitogen neuregulin 1 and its role in malignant peripheral nerve sheath tumors in the laboratory of Dr. Steven Carroll. She subsequently moved to Children's Hospital of Philadelphia where she pursued residencies in pediatrics and child neurology, serving as chief resident, followed by a neuro-oncology fellowship. She currently is an instructor of pediatrics at the Children's Hospital of Philadelphia in the Division of Oncology.



"Participation in the NFYIF has been wonderful with multifaceted benefits, including additional insight on future directions for my research, networking with other young investigators as well as established faculty in the field of NF research, and fostering potential collaborations."



"This award will be used to fund additional experiments on optimizing cellular migration, as well as to travel to a scientific meeting this spring."



2023 NFYIF Young Investigator Winners



# 2023 Junior Faculty Winners

## Clinical Scientist and Research Scientist

### First Runner-Up



#### **R. Taylor Sundby, MD**

*Assistant Research Physician  
National Cancer Institute  
Bethesda, Maryland*

#### **Cell-free DNA Fragmentomics Distinguish between Benign, Pre-malignant, and Malignant Peripheral Nerve Sheath Tumors in Neurofibromatosis Type 1**



"The NFYIF has established new collaborations and professional connections/support systems that are invaluable as I move forward as junior faculty."



"I will use this grant award to fund pilot sequencing. I am to build off the cfDNA fragmentomic work that I presented by building a bioinformatic platform to infer the methylome and transcriptome from fragmentomic features."

### Second Runner-Up



#### **Jodi Lukkes, PhD**

*Assistant Professor  
Indiana University School of Medicine  
Indianapolis, Indiana*

#### **Aberrant Cortico-striatal Neural Activity Underlies Impulsivity and ADHD in a Preclinical Model of Neurofibromatosis Type 1**



"The NFYIF helped my professional development with great networking opportunities and the chance to meet with leaders in the NF field."



"This grant award will go toward travel to the 2024 NF Conference in Belgium."



# 2023 Fellows Winners

## Clinical Fellows and Postdoctoral Researchers

### First Place



#### **Namrata Raut, PhD**

*Research Fellow*

*Cincinnati Children's Hospital Medical Center*

*Cincinnati, Ohio*

#### **Targeting Glial Cell Line–derived Neurotrophic Factor in Schwann Cells Reduces Pain-like Behaviors in a Pre-clinical Model of NF1**



"NFYIF is an amazing platform to present and get acquainted with the basic, translational, and clinical research in the field of Neurofibromatosis (NF). NFYIF also fosters collaboration, mentorship, and dialogues with NF patients, which is crucial for young investigator."



"I will use [the award] for travel to IASP 2024 World Congress of Pain."

### Second Place *[tie]*



#### **Lindy Zhang, MD**

*Clinical Fellow/PhD Graduate Student*

*Johns Hopkins University School of Medicine*

*Baltimore, Maryland*

#### **Investigating Mechanisms of Immune Escape in NF1-associated Peripheral Nerve Sheath Tumors**



"I have absolutely appreciated and thoroughly enjoyed the experience. It has helped me gain confidence in my oral presentation skills and answering questions regarding my research in a formal forum. This experience has taught me a lot about networking and making connections. Firstly, the mentors are world-renown and accomplished individuals, but to be able to sit down with them and ask them life questions and for professional advice makes me realize that they are also wonderful human beings, and I have gotten comfortable with being able to approach other professionals/mentors. Secondly, I have been able to connect with other young investigators at a variety of levels in training and backgrounds, and realize that I am not alone in some of my struggles and that we are all doing great work in the field."



"The tentative plan is to use the award to purchase a work laptop, so that I can have all the data analysis software available at one computer for easier accessibility."



## Second Place *[tie]*



### **Srirupa Bhattacharyya, PhD**

*Postdoctoral Research Fellow  
Massachusetts General Hospital  
Boston, Massachusetts*

#### **Preclinical Evaluation of the Third-generation, Bi-steric mTORC1-selective Inhibitor RMC-6272 in NF2-deficient Models**

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"This was my first year of attending NFYIF, and I found this to be a one-of-a-kind experience. It provided me the opportunity to present my work and to meet and learn from many experts and budding scientists in the NF field. Particularly, the round table discussions with mentors and fellow young investigators were enriching and insightful, sparking new ideas to pave the way for future scientific collaborations."

99

"I will use the grant award money to continue my research to develop NF2-associated tumor cell models. These models will be used to test compounds to ultimately discover new pathways that may lead to novel and effective treatment strategies for NF2."

## 2023 PhD Candidate Winner



### **Pernelle Pulh, MSc**

*PhD Student  
Mondor Institute for Biomedical Research  
Créteil, France*

#### **Decipher the Mechanisms Governing Cutaneous Neurofibromas Development in a Mouse Model of Neurofibromatosis Type 1**

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"Participating in the NFYIF provided me with the opportunity to present my research to other fellows in the NF field. The forum was inspiring and allowed me to network with faculty members as well as hear a powerful presentation from an NF patient advocate. I intend to keep all these experiences in mind as I move forward in my research."

99

"I plan to use the NFYIF grant award for the validation of single-cell RNA sequencing data on cutaneous neurofibromas sections."



## 2023 NFYIF Participants



**George Kwakye Annor, PhD**  
*Postdoctoral Trainee*  
*National Institutes of Health/National Cancer Institute*  
*Bethesda, Maryland*

Targeting DLK1 in In Vivo and In Vitro Tumor Models of MPNST with an Antibody–drug Conjugate Suggests DLK1 as a Druggable Target



**Srirupa Bhattacharyya, PhD**  
*Postdoctoral Research Fellow*  
*Massachusetts General Hospital*  
*Boston, Massachusetts*

Preclinical Evaluation of the Third-generation, Bi-steric mTORC1-selective Inhibitor RMC-6272 in NF2-deficient Models



**Stephanie Brosius, MD, PhD**  
*Instructor in Pediatrics*  
*University of Pennsylvania, Children's Hospital of Philadelphia*  
*Philadelphia, Pennsylvania*

Interneurons That BiTE: A Novel Cellular Therapy for High-grade Glioma



**Asmita Dhukhwa, PhD**  
*Post Doctoral Fellow*  
*The Johns Hopkins Wilmer Eye Institute*  
*Baltimore, Maryland*

Establishing an In Vitro Model to Study NF1 Pathology in Glial Cells Using Patient-derived iPSCs



**Jamie Grit, PhD**  
*Postdoctoral Fellow*  
*Van Andel Institute*  
*Grand Rapids, Michigan*

Ex Vivo Explant Model for Neurofibromatosis Type 1-related Cutaneous Neurofibromas



**Yang Hou, PhD**  
*Assistant Professor*  
*Florida State University*  
*Tallahassee, Florida*

Developmental Trajectories of Academic Function in Children with Neurofibromatosis Type 1: Demographic and Disease Predictors



**Maria Ioannou, MD**  
*Postdoctoral Research Fellow*  
*Johns Hopkins University*  
*Baltimore, Maryland*

MEK Inhibitor Mirdametinib Augments the Efficacy of Irradiation in NF1-deficient High-grade Glioma Preclinical Models



**Roope Kallionpää, PhD**  
*Senior Researcher*  
*University of Turku*  
*Turku, Finland*

The Effect of NF1 Germline and Somatic Alterations on Breast Cancer Phenotype and Survival



**Casey J. Keuthan, PhD**  
*Postdoctoral Fellow*  
*Johns Hopkins University*  
*Baltimore, Maryland*

Investigating the Molecular and Functional Effects of NF1 Mutations on Human Retinal Ganglion Cells



**Jodi Lukkes, PhD**  
*Assistant Professor*  
*Indiana University School of Medicine*  
*Indianapolis, Indiana*

Aberrant Cortico-striatal Neural Activity Underlies Impulsivity and ADHD in a Preclinical Model of Neurofibromatosis Type 1





## 2023 NFYIF Participants



**Vanessa Merker, PhD**

*Assistant Professor  
Massachusetts General Hospital  
Boston, Massachusetts*

Self-reported Access to Specialty Clinics and Receipt of Guideline-concordant Care among U.S. Patients with Neurofibromatosis 1: A National Survey of NF Registry Participants



**Béga Murray, PhD, MSc**

*Postdoctoral Fellow  
National Cancer Institute, National Institutes of Health  
Bethesda, Maryland*

SWI/SNF-associated DPFI Is a Unique Transcriptional Regulator of Malignant Peripheral Nerve Sheath Tumors



**Pernelle Pulh, MSc**

*PhD Student  
Mondor Institute for Biomedical Research  
Créteil, France*

Decipher the Mechanisms Governing Cutaneous Neurofibromas Development in a Mouse Model of Neurofibromatosis Type 1



**Jay Pundavela, PhD**

*Research Associate  
Cincinnati Children's Hospital Medical Center  
Cincinnati, Ohio*

CD8 T Cells: Fanning the Flame of Inflammation-driven Plexiform Neurofibroma Formation



**Namrata Raut, PhD**

*Research Fellow  
Cincinnati Children's Hospital Medical Center  
Cincinnati, Ohio*

Targeting Glial Cell Line-derived Neurotrophic Factor in Schwann Cells Reduces Pain-like Behaviors in a Pre-clinical Model of NF1



**R. Taylor Sundby, MD**

*Assistant Research Physician  
National Cancer Institute  
Bethesda, Maryland*

Cell-free DNA Fragmentomics Distinguish between Benign, Pre-malignant and Malignant Peripheral Nerve Sheath Tumors in Neurofibromatosis Type 1



**Jiawan Wang, PhD**

*Research Associate  
Johns Hopkins University School of Medicine  
Baltimore, Maryland*

CDK4/6 Inhibition Enhances SHP2 Inhibitor Efficacy and Is Dependent upon RB Function in Malignant Peripheral Nerve Sheath Tumors



**Lindy Zhang, MD**

*Clinical Fellow/PhD Graduate Student  
Johns Hopkins University School of Medicine  
Baltimore, Maryland*

Investigating Mechanisms of Immune Escape in NF1-associated Peripheral Nerve Sheath Tumors



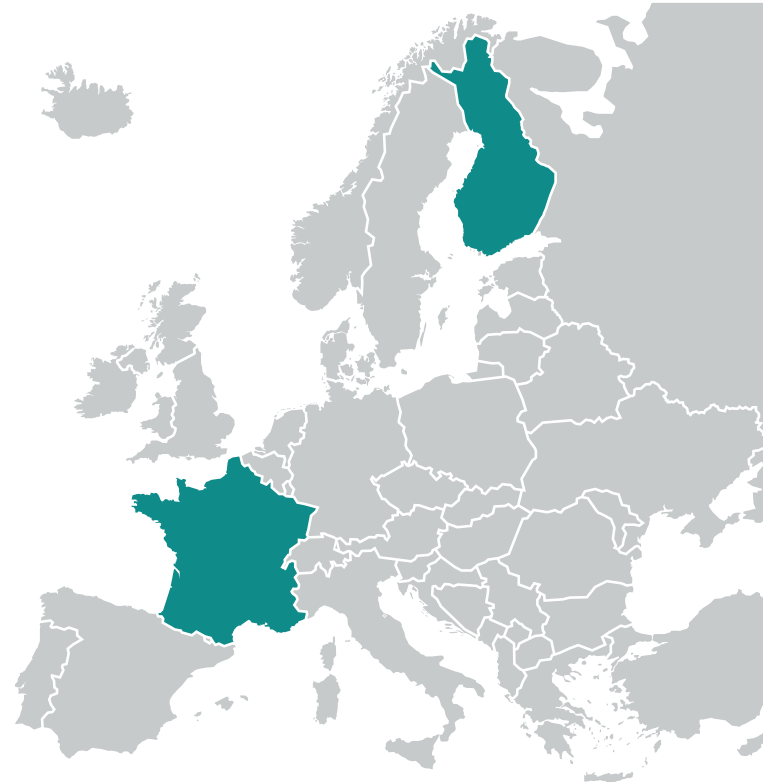
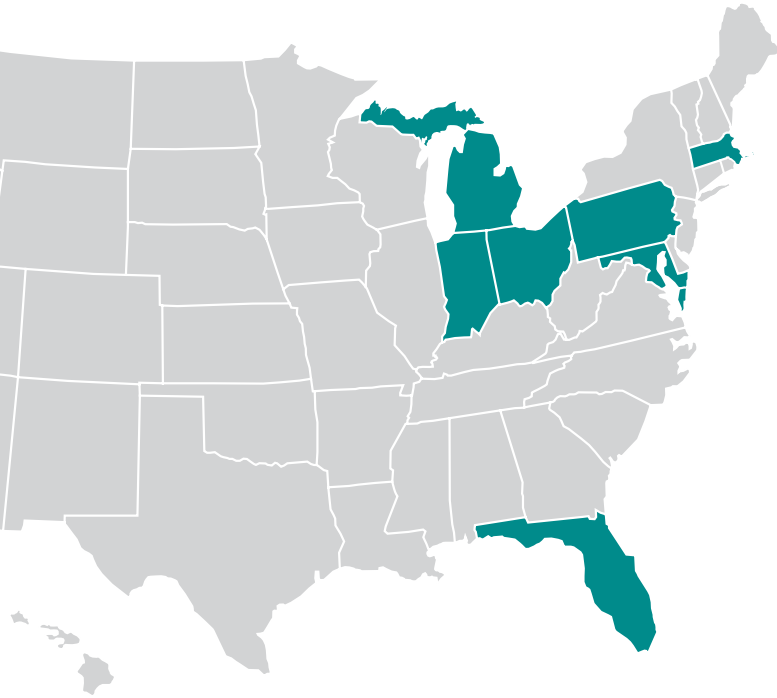
**Xiyuan Zhang, PhD**

*Staff Scientist  
National Cancer Institute  
Bethesda, Maryland*

A Comprehensive Algorithm to Predict Malignant Transformation of NF1 Nerve Sheath Tumors from Single-cell Transcriptomic Profiling



# At-a-Glance



Cincinnati Children’s Hospital Medical Center  
*Cincinnati, Ohio*

Florida State University  
*Tallahassee, Florida*

Indiana University School of Medicine  
*Indianapolis, Indiana*

Johns Hopkins University  
*Baltimore, Maryland*

The Johns Hopkins Wilmer Eye Institute  
*Baltimore, Maryland*

Massachusetts General Hospital  
*Boston, Massachusetts*

Mondor Institute for Biomedical Research  
*Paris, France*

National Cancer Institute,  
National Institutes of Health  
*Bethesda, Maryland*

National Cancer Institute,  
Pediatric Oncology Branch  
*Bethesda, Maryland*

University of Pennsylvania,  
Children's Hospital of Philadelphia  
*Philadelphia, Pennsylvania*

University of Turku  
*Turku, Finland*

Van Andel Institute  
*Grand Rapids, Michigan*

## YI ATTENDEES



PHD CANDIDATES



CLINICAL (MD)  
FELLOWS



POSTDOCTORAL (PhD)  
FELLOWS



JUNIOR FACULTY



# Faculty



2023 NFYIF Faculty



**Bruce R. Korf, MD, PhD**  
**(activity chair)**  
*Wayne H. and Sara Crews Finley  
 Endowed Chair in Medical Genetics  
 Distinguished Professor of Genetics  
 Associate Dean for Genomic Medicine  
 UAB Heersink School of Medicine  
 Chief Genomics Officer, UAB Medicine  
 Birmingham, Alabama*



**Andrea M. Gross, MD**  
**(activity co-chair)**  
*Assistant Research Physician  
 Pediatric Oncology Branch  
 National Cancer Institute  
 National Institutes of Health (NIH)  
 Bethesda, Maryland*



**Michael J. Fisher, MD**  
*Chief, Neuro-Oncology Section  
 Director, Neurofibromatosis Program  
 Hubert J.P. and Anne Faulkner  
 Schoemaker Endowed Chair in  
 Pediatric Neuro-Oncology  
 Professor of Pediatrics  
 Center for Childhood Cancer Research  
 and Division of Oncology  
 The Children's Hospital of Philadelphia  
 Philadelphia, Pennsylvania*



**AeRang Kim, MD, PhD**  
*Director of Clinical Research  
 Division of Oncology  
 Center for Cancer and Blood Disorders  
 Children's National Hospital  
 Associate Professor of Pediatrics  
 George Washington University School of  
 Medicine  
 Washington, DC*



**Eric Legius, MD, PhD**  
*Center of Human Genetics  
 University Hospital Leuven and  
 Catholic University of Leuven  
 Leuven, Belgium*



**Nancy Ratner, PhD**  
*Beatrice C. Lampkin Endowed Chair in  
 Cancer Biology  
 Professor of Pediatrics  
 Cincinnati Children's Hospital  
 Department of Pediatrics  
 University of Cincinnati College of  
 Medicine  
 Cincinnati, Ohio*



# 2023 NFYIF Highlights

## CTF MISSION MOMENT

### Patient Perspective

The NFYIF brought together some of the world's foremost NF thought leaders with a select group of high potential, high performing early-career NF researchers from across the United States; the result was the presentation of an immense amount of impactful NF science, formative professional and personal networking experiences, establishment of new peer-to-peer and peer-to-mentor relationships, and thus, an elemental shift in the trajectory of the NF field. And while those achievements are all crucially important, perhaps most important of all is actually the one thing undergirding everything else—the foundational “why” driving each and every person in attendance.

That “why” is, of course, the ultimate vision of improving care and optimizing outcomes for patients with neurofibromatosis. It is the empiric mission of the NFYIF and all those who attend, which is why young investigators called this year's CTF Mission Moment “the highlight of the whole conference.”

The CTF Mission Moment gave the stage to the 2023 CTF Patient Ambassador, Michele Holbrook, and allowed her to share her personal NF journey. Ms. Holbrook inspired, informed, and actuated everyone in the room, effectively humanizing the science and leaving us all in awe of her strength, courage, and determination. The impact Ms. Holbrook had on the NFYIF was both tangible and intangible—immeasurable in the best possible way—and we very much hope to have her and many other patients/caregivers share their stories at future installments of the NFYIF.



Patient Advocate, Michele Holbrook



"We envision a day when patients can live their lives free of the pain and difficulties that come with nerve tumors, and our innovative team-based approach to drug development is making that vision a reality."





## Educational Highlights

### Mentoring Moments

Guided by the overarching mission to cultivate, inform, and empower young neurofibromatosis researchers, the NFYIF provided an intimate setting in which our young investigators were privy to Mentoring Moments sessions with our Expert Judge Mentors, all of whom are experienced and pre-eminent thought leaders in the field. Within these mentoring sessions, young investigators were offered actionable, real-world, tried-and-true advice on circumventing the prominent obstacles faced during early-career inflection points. During our opening Mentoring Moments session, our Expert Judge Mentor panel discussed personal experiences illustrating the power of networking and community-building, and shared practical pearls for navigating the complex task of translating clinical concepts into funded protocols in the initiation and conductance of clinical trials. Perhaps most notably, our young investigators were granted unprecedented access to top NF Key Opinion Leaders and were provisioned one-on-one networking opportunities with peers, both of which possess the power to fundamentally alter their career trajectory and bolster future research productivity.

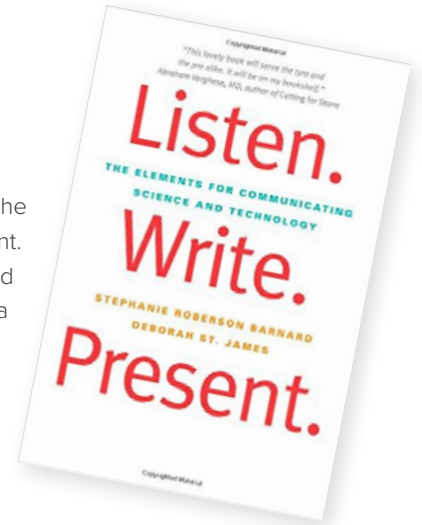




# Educational Highlights

## Presentation Skills Enhancement Workshop

To augment the professional development aspects of the NFYIF, 2023 participants had the opportunity to receive individual coaching sessions with an expert from Listen Write Present. Young investigators who attended the one-on-one pre-program coaching sessions received expert advice and critique of their presentation and public speaking skills, and were given a copy of the book *Listen. Write. Present.* In addition, the expert coaches provided participants with tips for effective navigation of expert Q&A and research defense.



## 2023 NFYIF Professional Scientific Communication Coaches



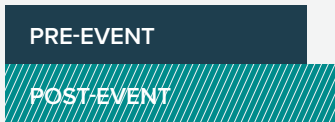
**Stephanie Roberson Barnard**  
*Listen Write Present, LLC*  
*Greensboro, North Carolina*



**Paul Casella**  
*Health Care Communications Group*  
*University of Iowa Carver College of Medicine*  
*Iowa City, Iowa*



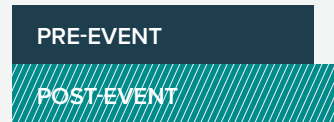
“Participating in the NFYIF bolstered my communication skills and confidence in presenting my research to colleagues. I hope it will have a positive impact on my ability to secure research funding in the near future.”



Following coaching and abstract presentations, average participant confidence in their ability to present scientific information to peers increased from

**3.14 to 3.43\***

*\* on a 4 point scale*



Similarly, average participant confidence in ability to defend their research following the event increased from

**3.14 to 3.43\***

*\* on a 4 point scale*



## HOW ATTENDING THE SUMMIT WILL Impact Young Investigators' Careers



"I discussed ideas for potential research collaborations with two other young investigators, and I was able to network with young investigators and judges in my field, increasing awareness of my research and skillset among this audience, which may benefit me down the line."



"I have expanded my network of potential collaborators and mentors in the NF community."



# Updates from the 2022 NFYIF Participants

## Research Publications, National Meeting Presentations, and Honors and Awards

The NFYIF is a highly competitive research and professional development forum that strives to encourage, promote, and empower young investigators to forge collegial connections and acquire the necessary skills and relationships to increase their research productivity and catalyze their career trajectory. The following section is a glimpse at their accomplishments and research, and is interwoven with prior participant reflections about the impact that participating in the forum has had on their professional lives since attending the last meeting in 2022.

### Publications

**Banerjee J**, Friedman JM, Klesse LJ, et al. COVID-19 in people with neurofibromatosis 1, neurofibromatosis 2, or schwannomatosis. *Genet Med*. 2023;25(2):100324.

**Banerjee J**, Taroni JN, Allaway RJ, et al. Machine learning in rare disease. *Nat Methods*. 2023;20(6):803–814.

Douglass JD, Ness KM, Valdearcos M, [...] **Banerjee J**, et al. Obesity-associated microglial inflammatory activation paradoxically improves glucose tolerance. *Cell Metab*. 2023;35(9):1613–1629.e8.

Reese JT, Blau H, Casiraghi E, [...] **Banerjee J**, et al. Generalisable long COVID subtypes: findings from the NIH N3C and RECOVER programmes. *EBioMedicine*. 2023;87:104413.

**Laraba L**, Hillson L, de Guibert JG, et al. Inhibition of YAP/TAZ-driven TEAD activity prevents growth of NF2-null schwannoma and meningioma. *Brain*. 2023;146(4):1697–1713.

Ly KI, **Merker VL**, Cai W, et al. Ten-year follow-up of internal neurofibroma growth behavior in adult patients with neurofibromatosis type 1 using whole-body MRI. *Neurology*. 2023;100(7):e661–e670.

**Merker VL**, Gross AM, Widemann BC, Plotkin SR. Advancing neurofibromatosis and schwannomatosis clinical trial design: consensus recommendations from the Response Evaluation in Neurofibromatosis and Schwannomatosis (REINS) international collaboration. *Clin Trials*. 2023:17407745231201345.

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Santoro SL, Cabrera MJ, Haugen K, Krell K, **Merker VL**. Indicators of health in Down syndrome: a virtual focus group study with patients and their parents. *J Appl Res Intellect Disabil*. 2023;36(2):354–365.

Wolters PL, Ghriwati NA, Baker M, [...] **Merker VL**, et al. Perspectives of adults with neurofibromatosis regarding the design of psychosocial trials: results from an anonymous online survey. *Clin Trials*. 2023:17407745231209224.

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Özden C, Mautner VF, Farschtschi S, Molwitz I, **Ristow I**, et al. Asymmetry of thalamic hypometabolism on FDG-PET/CT in neurofibromatosis type 1: association with peripheral tumor burden. *J Neuroimaging*. 2024;34(1):138–144.

Riedel C, **Ristow I**, Lenz A, et al. Validation of 4D flow cardiovascular magnetic resonance in TIPS stent grafts using a 3D-printed flow phantom. *J Cardiovasc Magn Reson*. 2023;25(1):9.

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**Ristow I**, Riedel C, Lenz A, et al. Current imaging strategies in patients with abdominal aortic aneurysms. *Rofa*. 2024;196(1):52–61.





- Wright F, Warncke M, Sinn M, **Ristow I**, et al. Assessment of aortic diameter in Marfan patients: intraindividual comparison of 3D-Dixon and 2D-SSFP magnetic resonance imaging. *Eur Radiol*. 2023;33(3):1687–1697.
- Tintelnot J, **Ristow I**, Sauer M, et al. Translational analysis and final efficacy of the AVETUX trial—avelumab, cetuximab and FOLFOX in metastatic colorectal cancer. *Front Oncol*. 2022;12:993611.
- Blakeley JO, Le LQ, Lee SY, [...] **Romo CG**, et al. A call for discovery and therapeutic development for cutaneous neurofibromas. *J Invest Dermatol*. 2023;143(8):1351–1357.
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- Fertitta L, Sarin KY, Bergqvist C, [...] **Romo CG**, et al. cNF-skindex in adults living with neurofibromatosis 1: severity strata in France and validation in United States adults. *J Invest Dermatol*. 2023;143(11):2226–2232.e1.
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- Ly I, **Romo CG**, Gottesman S, et al. Target product profile for cutaneous neurofibromas: clinical trials to prevent, arrest, or regress cutaneous neurofibromas. *J Invest Dermatol*. 2023;143(8):1388–1396.
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“Attending NFYIF and especially networking in-person with the other young investigators was an amazing experience. Working in a rare disease, there are often few other NF researchers at our home institutions or cities, so NFYIF was a crucial part of building a sense of community. While attending NFYIF, I was able to brainstorm two new research collaborations with other young investigators, faculty, and representatives from two of the partner organizations. And since NFYIF, I have kept in touch with many other NFYIF attendees for special projects and to spend time together at other professional meetings.”

—Vanessa Merker, PhD



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“After attending the NFYIF 2022, I have become part of a superb network of young investigators, which has led to collaborative projects and strengthened my desire to continue my career in NF research.”

—Liyam Laraba, PhD

## Collaborative Publications among NFYIF 2022 Young Investigators

**Ioannou M**, Zhang L, Schatz K, et al. Plexiform neurofibroma of the liver, with malignant transformation to MPNST, in a pediatric patient without neurofibromatosis type 1. *Neurooncol Adv.* 2023;5(1):vdad125.



## Presentations

### *Children's Tumor Foundation (CTF) Neurofibromatosis (NF) Conference 2023*

June 24–27, 2023; Scottsdale, Arizona

- Acar S**, Bhatia H, Bocherding D, et al. *UBR5*, a chromosome 8 gene, regulates cell survival in MPNST. Poster 1.
- Banerjee J**, Grande BM, Vu AN, et al. Maximizing utility of neurofibromatosis genomic data through the NF data portal and cBioportal. Poster 11.
- Bouley SJ**, Fernandez F, Wallace P, et al. Platform: Targeting the NF-kappaB pathway to treat *NF1*-deficient tumors. Oral Session Presentation.
- Ioannou MI**, Lalwani K, Ayanlaja A, et al. MEK inhibitor mirdametininib augments the efficacy of irradiation in *NF1* deficient high-grade glioma preclinical models. Poster 61.
- Laraba L**, Blake J, de Assis L, et al. Platform: TEAD autoparmitoylation inhibitors prevent *NF2*-deficient meningioma growth in an *in vivo* skull convexity model. Oral Session Presentation.
- Merker VL**. Diagnostic odyssey in schwannomatosis. Oral Session Presentation.
- Pundavela J**, Dinglasan SA, Touvron M, et al. Contribution of sterile inflammation through STING and T cells to plexiform neurofibroma initiation and growth. Poster 95.
- Raut NGR**, Adlakha A, Maile LA, et al. Schwann cell calcium and growth factor signaling modulates pain in *NF1*. Poster 99.
- Ristow I**, Apostolova I, Kaul MG, et al. Differentiation of peripheral nerve sheath tumors in *NF1* – intraindividual comparison of the diagnostic accuracy of diffusion-weighted MRI and <sup>18</sup>F-FDG-PET/CT. Poster 116.
- Romo CG**, Blakeley JO, Roberts J, et al. Platform: Detection of distinct nodular lesions on whole-body MRI in a high-risk population with neurofibromatosis type 1. Oral Session Presentation.
- Sundby RT**, Szymanski JJ, Pan AC, et al. Platform: Early detection of malignant, pre-malignant and benign peripheral nerve sheath tumors with liquid biopsy cell-free DNA fragmentomics. Oral Session Presentation.
- Williams KB**, Sverak H, Galvin R, et al. Study of nutraceutical intervention with high phenolic extra virgin olive oil and curcumin for neurofibromatosis type 1. Poster 129.
- Zhang L**, Lemberg K, Calizo A, et al. A retrospective analysis of treatment sequence and outcomes in patients with relapsed MPNST. Poster 152.
- Zhang L**, Maalouf A, Pollard K, et al. Mechanisms of immune escape in *NF1*-associated peripheral nerve sheath tumors. Poster 137.
- Zhang X**, Abbas S, Syed N, et al. Platform: Single-cell sequencing reveals transcriptomic diversity that facilitates the malignant transformation of *NF1* nerve sheath tumors. Oral Session Presentation.

“

I am extremely grateful for the opportunity to have participated in the NFYIF. While I have had the opportunity to network at various conferences, I think that the NFYIF provides a unique opportunity for new investigators, whether it be clinicians or researchers, to network with those at the same career stage as they are, thus providing the foundation for future collaborations. It is also heartening to see support equally distributed between non-profit and for-profit groups, as I believe that the future of NF research will rely heavily on academic-industry collaborations. I have encouraged numerous early-career stage NF researchers to participate in future NFYIF meetings, and one will be at this year's meeting. I think that, in order for researchers to remain in the NF field, they need consistent support at every stage from those within the field who possess the knowledge and experience to guide new researchers, and having prominent members of the NF community participate in the NFYIF as mentors and judges is an excellent way to do this. Overall, I'm extremely thankful for the opportunities participating in the NFYIF has provided me with, and I look forward to attending again in the future.”

—Stephanie Joy Bouley, PhD



*7th Annual Children's Cancer Foundation Research Symposium 2023*

*June 14, 2023; Greenbelt, Maryland*

**Sundby, RT.** Cell-free DNA fragmentomics distinguish between benign, pre-malignant and malignant peripheral nerve sheath tumors in neurofibromatosis type 1. Oral Presentation.

## Honors and Awards

**Stephanie Joy Bouley, PhD**

2023 | Early Investigator Research Award, Neurofibromatosis Research Program (NFRP)

**Vanessa Merker, PhD**

2023 | Funding from the Patient Centered Outcome Research Institute

**Xiyuan Zhang, PhD**

2023 | Special Act Award, National Cancer Institute

2023 | Young Investigator Travel Award, Strategic Advances in Sarcoma Science

# Start Planning for 2024!



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## NEUROFIBROMATOSIS

Young Investigators' Forum

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