

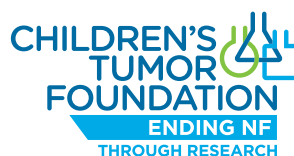


— 4<sup>TH</sup> ANNUAL —

# 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,

The fourth Neurofibromatosis Young Investigator's Forum (NFYIF) was held in Baltimore, Maryland, on December 6, 2024. As in past years, 20 pre-and post-doctoral trainees and junior faculty, selected after a competitive process, presented brief talks on their research before a panel of Expert Judge Mentors. Awards were issued to the highest scoring talks in several categories based on level of training and whether the talk focused on clinical or laboratory science. In addition, the Mentors offered advice to the attendees, including career guidance and ways to help advance their research on neurofibromatosis. There were lively question and answer sessions after each talk, with participation both from the judges and the audience. Exciting progress continues to be made in our understanding of neurofibromatosis that is leading to improved means of diagnosis and treatment. That the future of neurofibromatosis research is in good hands was abundantly evident from the enthusiasm of the attendees and the high quality of the scientific presentations.

We congratulate all the young investigators who took part in the 2024 NFYIF, both for the quality of their research and for their commitment to helping end the burden of neurofibromatosis for patients and their families. It is events like this that justify optimism for continued advances on behalf of all those who live with neurofibromatosis every day.

Sincerely,

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

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



2024 NFYIF Faculty & Young Investigators



"It is great to be here and see young people getting started in the NF field. They bring new ideas, get me excited about what I'm doing—but really, I want to be here to foster their growth, because we need new, young, smart people in the field if we are going to solve NF."



## 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, fellows, postdoctoral researchers, and PhD students 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 20 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 fourth annual NFYIF installment robustly achieved this goal, and in so doing, effectively laid the groundwork for future successes.



CECONCEPTS  
**NEUROFIBROMATOSIS**  
Young Investigators' Forum



# Distinguished Young Investigator

Each year, the top researcher and presenter in the Junior Faculty category is presented with the Neurofibromatosis Distinguished Young Investigator Research Award, which represents excellence, commitment, and promise in the field of neurofibromatosis research.

## 2024 Neurofibromatosis Distinguished Young Investigator Research Award



### Chelsea Kotch, MD, MSCE

*Attending Physician/Assistant Professor  
Children's Hospital of Philadelphia  
Philadelphia, Pennsylvania*

### Analysis of Treatment Approach and Outcomes in Children and Adults with Neurofibromatosis Type 1-associated High-grade Glioma

Chelsea Kotch is a pediatric neuro-oncologist at the Children's Hospital of Philadelphia and assistant professor of pediatrics at the University of Pennsylvania who cares for children and young adults in the Neuro-Oncology and NF Multi-Disciplinary Clinics. She is a clinical researcher and Francis S Collins Scholar in NF Translational and Clinical Research with a focus on improving outcomes for children with NF1-associated tumors through advanced epidemiologic methods. She is also a study chair in the NF Clinical Trials Consortium.

36

"Participating in the NFYIF provided me with the opportunity to network and interact with experts in the field of NF1 and to receive feedback on my research program and career goals. During the NFYIF conference, I was able to hear the expert investigators describe challenges they encountered during their career development and methods to mitigate such challenges; these conversations were of particular importance to me as I begin to establish my independent research program."

99

"I will use the funding from this award to support my developing independent research program; specifically, it is with these funds that I will obtain additional advanced informatics support for data analysis of outcomes of children with NF1-associated tumors through CHOP's Biostatistics and Database Management core."



2024 NFYIF Young Investigator Winners



# 2024 Fellows Winners

## Postdoctoral Researchers

### First Place



#### Marie-Lena Schmalhofer, MD

*Third-year Radiology Resident  
Universitätsklinikum Hamburg-Eppendorf  
Hamburg, Germany*

#### Enhancing Neurofibroma Segmentation in Whole-body MRI Leveraging an Anatomy-informed Dynamic UNet-based Approach in Patients with Neurofibromatosis Type 1

66

"Participating in the NFYIF and experiencing the presentations on current research priorities was a very special and unique experience for me. The forum provided diverse insights into new, exciting, and innovative research topics in the field of neurofibromatosis and enhanced my ability to engage in valuable and constructive discussions with international experts. These interactions sharpened my ability to critically evaluate my research to address specific challenges in neurofibromatosis research. Additionally, the mentoring and speech training improved my presentation skills and helped me build confidence in effectively presenting research findings at international events. The forum was a valuable platform for interdisciplinary networking and interdisciplinary exchange with leading experts in the field, exchanging ideas, and gathering inspiration for new research endeavors. This experience has undoubtedly enriched both my personal and professional development as a researcher, leaving a lasting impact on my career trajectory."

”

"I plan to use the prize money to support further research projects with a focus on whole-body MRI (WB-MRI) in neurofibromatosis. For example, the funds can be used to finance specific image analysis software, for interdisciplinary collaborations, or to support patient recruitment and data collection. In this way, we hope to improve diagnosis and monitoring strategies for people with neurofibromatosis."





## Second Place



### Alex Dyson, PhD

*Postdoctoral Research Fellow  
Massachusetts General Hospital  
Boston, Massachusetts*

### MEK Inhibition as a Potential Therapeutic Strategy for the Non-tumor Manifestations of Neurofibromatosis Type 1 (NF1)



"I was hugely grateful to receive insights into my research from experts in the NF field that will certainly shape the direction, and improve the quality, of my work. Equally, I was fascinated to hear their opinions on contemporary topics within the field, especially regarding therapies. But most importantly, it was invaluable to have the opportunity to meet other early NF researchers, with the possibility of collaboration in the future."



"This award will fund my travel to (and registration for) research conferences, including the 66th Annual Drosophila Research Conference (San Diego, March 2025) and the Neurobiology of Drosophila meeting (Cold Spring Harbor, October 2025)."

## 2024 PhD Candidate Winners

### First Place



### Francesca Kovaci, MSc

*PhD Student  
Institut Mondor of Biomedical Research  
Créteil, France*

### Spine Deformity in Mice Lacking NF1 Gene in Boundary Cap-derived Osteoblasts



"Participating in the NFYIF has been an incredible experience for me—impactful on my professional development. This forum gave me the opportunity to present my findings, exchange ideas, and gain fresh perspectives, which are essential for advancing my work. I especially loved the opportunity to connect and engage with experts in the field, whose feedback on my research work was inspiring. These interactions also reinforced my desire to continue as a post-doctoral fellow in neurofibromatosis research with greater enthusiasm. Hopefully, I will contribute meaningfully to the field of NF research."



"I plan to use the award to further support my career, to participate in the conferences, and to do bioinformatic courses."



## Second Place



### Zoe Cappel, BA

*PhD Student*

*Cincinnati Children's Hospital Medical Center*

*Cincinnati, Ohio*

### **Molecular and Circuit Mechanisms of Visual Hypersensitivity in Neurofibromatosis Type 1 Model Mice**

"In attending NFYIF, I was able to present my research to an audience outside of my institution for the first time, allowing me to take leadership of my work and receive feedback from experts in the field and fellow peers.



However, the sense of community that I felt at NFYIF was perhaps the most impactful aspect for me. Being immersed in such a welcoming and collaborative environment allowed me to visualize the role that I could fill within the NF community and the difference that I might be able to make through my work."



"I plan to put this money toward traveling to future NF meetings and other scientific conferences in hopes of expanding my scientific communication and critical thinking skills as I grow into an independent neuroscientist."



# 2024 NFYIF Young Investigator Presenters



## Stephanie Bouley, PhD

*Research Fellow  
Massachusetts General Hospital  
Boston, Massachusetts*

Disrupting Signaling Networks of NF1-deficient Tumors to Identify Novel Therapeutic Pathway Targets



## Zoe Cappel, BA

*PhD Student  
Cincinnati Children's Hospital Medical Center  
Cincinnati, Ohio*

Molecular and Circuit Mechanisms of Visual Hypersensitivity in Neurofibromatosis Type 1 Model Mice



## Urania Dagalakis, MD, MHSc

*Clinical Fellow  
National Cancer Institute  
Bethesda, Maryland*

Left Ventricular Ejection Fraction Changes over Time in Children and Adults with Neurofibromatosis Type 1 (NF1) on Clinical Trials with Selumetinib for Inoperable Plexiform Neurofibromas



## Alex Dyson, PhD

*Postdoctoral Research Fellow  
Massachusetts General Hospital  
Boston, Massachusetts*

MEK Inhibition as a Potential Therapeutic Strategy for the Non-tumor Manifestations of Neurofibromatosis Type 1 (NF1)



## Chelsea Kotch, MD, MSCE

*Attending Physician/Assistant Professor  
Children's Hospital of Philadelphia  
Philadelphia, Pennsylvania*

Analysis of Treatment Approach and Outcomes in Children and Adults with Neurofibromatosis Type 1-associated High-grade Glioma



## Franceska Kovaci, MSc

*PhD Student  
Institut Mondor of Biomedical Research  
Créteil, France*

Spine Deformity in Mice Lacking NF1 Gene in Boundary Cap-derived Osteoblasts



## Dan Liu, PhD

*Postdoctoral Scholar  
Florida State University  
Tallahassee, Florida*

Age Trends of Internalizing and Externalizing Problems in Children and Adolescents with Neurofibromatosis Type 1: Integrative Analysis of Data from Nine Institutions



## Jodi Lukkes, PhD

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

Investigating the Role of NF1 in Social Behavioral Deficits Utilizing a Preclinical Model of Neurofibromatosis Type 1



## Clara Nogué i Ansón, MSc

*PhD Student  
Bellvitge Biomedical Research Institute (IDIBELL)  
Barcelona, Spain*

Dissecting the Molecular Mechanisms Driving DGCR8-associated Schwannomatosis



## Sara Ortega Bertran, MS

*PhD Student  
Instituto de Investigación Biomédica de Bellvitge (IDIBELL)  
Barcelona, Spain*

Using a Pre-clinical Platform for MPNST Precision Oncology and Personalized Medicine





# 2024 NFYIF Young Investigator Presenters



**Gorkem Oztosun, MD**  
*Postdoctoral Research Fellow*  
*Washington University–St. Louis*  
*St. Louis, Missouri*  
 Generation of a Chromosome 8 Gain;NF1-/- hiPSC–derived Schwann Cell Precursor Model



**Marie-Lena Schmalhofer, MD**  
*Third-year Radiology Resident*  
*Universitätsklinikum Hamburg-Eppendorf*  
*Hamburg, Germany*  
 Enhancing Neurofibroma Segmentation in Whole-body MRI Leveraging an Anatomy-informed Dynamic UNet-based Approach in Patients with Neurofibromatosis Type 1



**James (Jerod) Sears, MD**  
*Clinical Fellow*  
*Washington University–St. Louis*  
*St. Louis, Missouri*  
 Avotemetinib and Defactinib as Combination Therapy in Chromosome 8 Gain-associated Malignant Peripheral Nerve Sheath Tumors (MPNST)



**Alexis Stillwell, BS**  
*PhD Student*  
*Pennington Biomedical Research Center*  
*Baton Rouge, Louisiana*  
 Developmental Analyses of Skeletal Manifestations in Knock-in Mouse Model of Neurofibromatosis Type 1 p.M992del“Mild” Patient Mutation



**Bavani Subramaniam, PhD**  
*Research Postdoctoral Fellow*  
*Children's National Hospital*  
*Washington, DC*  
 Targeting PRMT5 in MTAP-deleted NF1 High-grade Gliomas



**Daochun Sun, PhD**  
*Assistant Professor*  
*Medical College of Wisconsin*  
*Milwaukee, Wisconsin*  
 Stem-like Cells Hijack SPP1 Signaling to Program Macrophages in NF1 Tumor Progression



**Jack Thornton, PhD**  
*Postdoctoral Research Fellow*  
*Johns Hopkins*  
*Baltimore, Maryland*  
 Development of a Microglia-integrated Brain Organoid Model for Studying Neurofibromatosis Type 1



**Grace Yang, BS**  
*Undergraduate Research Assistant*  
*Stanford University*  
*Stanford, California*  
 Analyzing Cognitive and Behavioral Profiles of NF1 using IQ-Matched Controls

## Alumni Presenters



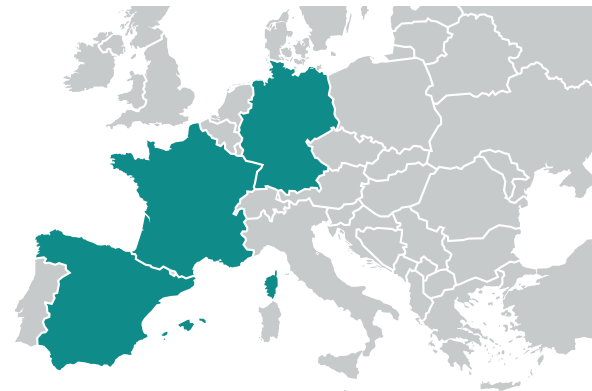
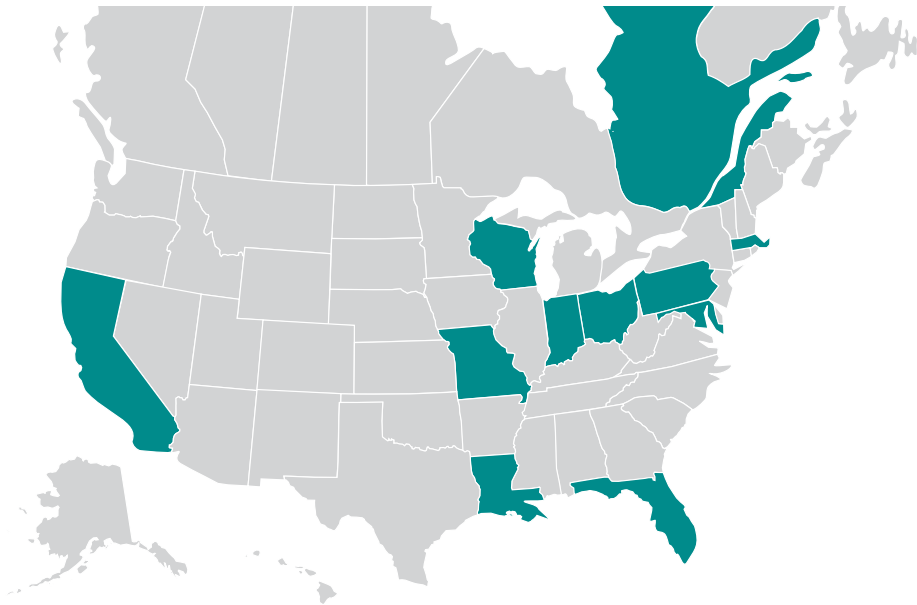
**Vanessa Merker, PhD**  
*Assistant Professor*  
*Massachusetts General Hospital/*  
*Harvard Medical School*  
*Boston, Massachusetts*  
 Development and Initial Feasibility Testing of an Online Platform to Promote Evidence-based Care for Underserved Patients with Neurofibromatosis 1 (NF1)



**Russell (Taylor) Sundby, MD**  
*Assistant Research Physician*  
*National Institutes of Health, Pediatric Oncology Branch*  
*Bethesda, Maryland*  
 NF1 Single-Cell Tumor Atlas Informs an Accurate, Non-invasive Circulating Proteomic Assay



# At-a-Glance



Bellvitge Biomedical Research  
Institute (IDIBELL)  
*Barcelona, Spain*

Children's Hospital of Philadelphia  
*Philadelphia, Pennsylvania*

Children's National Medical Center  
*Washington, DC*

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

Centre Hospitalier  
Universitaire de Sherbrooke  
*Sherbrooke, Quebec*

University Medical Center  
Hamburg-Eppendorf  
*Hamburg, Germany*

Florida State University  
*Tallahassee, Florida*

Medical College of Wisconsin  
*Milwaukee, Wisconsin*

Mondor Institute for  
Biomedical Research  
*Créteil, France*

Indiana University  
School of Medicine  
*Indianapolis, Indiana*

Johns Hopkins University  
*Baltimore, Maryland*

Massachusetts General Hospital  
*Boston, Massachusetts*

National Institutes of Health,  
Pediatric Oncology Branch  
*Bethesda, Maryland*

Pennington Biomedical  
Research Center  
*Baton Rouge, Louisiana*

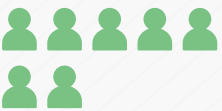
Stanford University  
*Palo Alto, California*

Washington University—St. Louis  
*St. Louis, Missouri*

## 27 PARTICIPANTS



YOUNG INVESTIGATORS

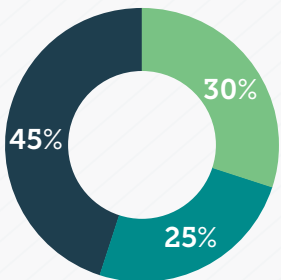


EXPERT JUDGE MENTORS



PATIENT ADVOCATES

## YOUNG INVESTGATORS



- FELLOW/POST DOCTORAL RESEARCHER
- JUNIOR FACULTY
- PHD/MD CANDIDATE



## 2024 NFYIF Expert Faculty Judges



2024 NFYIF Faculty



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

*Professor of Genetics  
University of Alabama–Birmingham  
Birmingham, Alabama*



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

*Assistant Research Physician  
Pediatric Oncology Branch  
National Cancer Institute  
National Institutes of Health  
Bethesda, Maryland*



**Thomas De Raedt, PhD**

*Beatrice C. Lampkin  
Endowed Chair in Cancer Biology  
Assistant Professor  
Children's Hospital of Philadelphia  
University of Pennsylvania  
Philadelphia, Pennsylvania*



**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**

*Emeritus Professor  
Catholic University of Leuven  
Leuven, Belgium*



### **Nancy Ratner, PhD**

*Beatrice C. Lampkin Endowed Chair in Cancer Biology  
Program Leader, Cancer Biology and Neural Tumors Program  
Co-Director, Rasopathy Program  
Professor of Pediatrics, Division of Experimental Hematology and Cancer Biology  
Cancer and Blood Diseases Institute  
Cincinnati Children's Hospital Medical Center  
Cincinnati, Ohio*

## Patient Advocates



### **Evan Neale**

*Safety and Leadership Speaker  
Declan's Storyteller*

### **Sarah Neale**

*Mother to NF Warrior, Declan*





## 2024 NFYIF Highlights

### CTF MISSION MOMENT

### Patient and Caregiver Perspectives

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.



CTF & Patient Advocates

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 Evan and Sarah Neale and allowed them to share their NF journey as the parents of Declan, who faces his own NF1 journey with the unwavering support of his two brothers and sister. Mr. Neale inspired, informed, and enthralled everyone in the room, effectively humanizing the science and leaving us all in awe of the entire family's strength, courage, and determination. The impact the Neales had on the NFYIF was both tangible and intangible—immeasurable in the best possible way—and we very much hope to have many other patients/caregivers share their stories at future installments of the NFYIF.



Having patient advocates at the NFYIF brings heart and a human connection to the science, showing how research impacts real lives. Advocacy can bridge the gap between data and a patient's reality, reminding everyone of the ultimate goal: improving life for patients and families dealing with NF.

We left with a profound sense of hope. Listening to the attendees at the meeting felt like piecing together a massive puzzle—each thought and idea brings us closer to a clearer, brighter future for those with NF. This experience motivates us to share the promising advancements with others, highlighting the dedication behind this cause. We are committed to elevating our efforts in raising awareness, increasing funding, and inspiring collective action to drive progress forward.

*Evan and Sarah Neale*

[Click here to watch Evan Neale's message to the 2024 Young Investigators](#)





## 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.



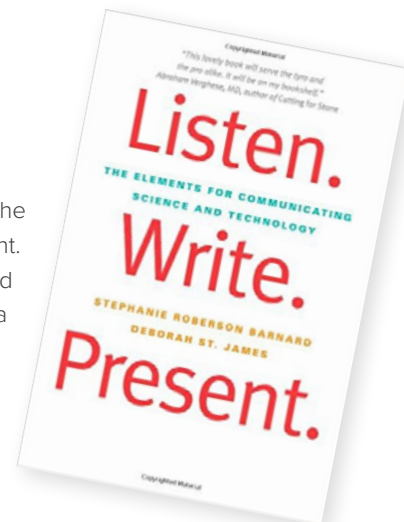




## Educational Highlights

### Presentation Skills Enhancement Workshop

To augment the professional development aspects of the 2024 NFYIF, 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 coach provided participants with tips for effective navigation of expert Q&A and research defense.



### 2024 NFYIF Professional Scientific Communication Coach



**Stephanie Roberson Barnard (Coach)**

*Listen Write Present LLC  
Greensboro, North Carolina*

PRE-EVENT

POST-EVENT

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

**2.62 to 3.33\***

*\* on a 4 point scale*

PRE-EVENT

POST-EVENT

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

**2.54 to 2.83\***

*\* on a 4 point scale*

36

"I greatly appreciate your suggestions, which helped me make significant improvements... I've learned a lot about how to enhance my future presentations."





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

Prior to the forum, Young Investigators were asked what they hoped to gain from attending NFYIF. One wrote that they hoped to gain "a stronger confidence with presenting scientific data." Another expressed that they wanted "exposure to and experience with dissemination of research findings." A third mentioned that they saw the forum as "an opportunity to gain insights from leading experts, foster meaningful collaborations, and address key challenges in NF research." At the conclusion of the forum, 100% of respondents stated that participating in NFYIF will impact their current research and/or professional career, with noted gains relating to projects, collaborations, and faculty advice.

### Overall Forum Comments



### Most Useful Information Gained





# Updates from the 2023 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 since attending the last meeting in 2023.

### Publications

- Ukhyun J, **Annor GK**, Shern JF. Abstract LB404: ATR inhibitors synergize with DHFR inhibitors in rhabdomyosarcoma cells by disrupting DNA damage checkpoint. *Cancer Res.* 2024;84(7\_Suppl):LB404.
- Bhattacharyya S**, Oblinger JL, Beauchamp RL, et al. Preclinical evaluation of the third-generation, bi-steric mechanistic target of rapamycin complex 1-selective inhibitor RMC-6272 in NF2-deficient models. *Neurooncol Adv.* 2024;6(1):vdae024.
- Brosius SN**, Manley W, Harvey K, et al. HGG-49. Interneurons that bite: a cellular delivery system for pediatric high-grade glioma. *Neuro-Onc.* 2024;26(Suppl 4):0.
- Harvey K, Labella K, Liou A, **Brosius S**, De Raedt T. Spheroid drug sensitivity screening in glioma stem cell lines. *J Vis Exp.* 2024;(204):10.3791/65655.
- Shin J, Zhang A, Meyer A, [...] **Brosius S**, et al. LGG-55. Differential toxicity profile of individual MEK inhibitors: a multi-institutional retrospective review. *Neuro-Onc.* 2024;26(Suppl 4):0.
- Brosius S**, Manley W, Anderson S, De Raedt T. EXTH-29. Interneurons that bite, a cellular therapy for high grade glioma. *Neuro-Onc.* 2023;25(Suppl 5):v230.
- Cochrane GD, Anwar SD, Weaver AN, **Brosius SN**, et al. A continuing clinical education course to maintain clinical competencies and foster new clinical knowledge during the graduate school years of MD-PhD training. *J Clin Transl Sci.* 2023;7(1):e270.
- Kotch C, **Brosius SN**, De Raedt T, Fisher MJ. Updates in the management of central and peripheral nervous system tumors among patients with neurofibromatosis type 1 and neurofibromatosis type 2. *Pediatr Neurosurg.* 2023;58(5):267–280.
- Britz J, Ojo E, Haque N, **Dhukhwa A**, et al. Sex-dependent effects of chronic circadian disruption in AβPP/PS1 Mice. *J Alzheimers Dis.* 2024;97(2):855–870.
- Grit JL**, McGee LE, Tovar EA, et al. p53 modulates kinase inhibitor resistance and lineage plasticity in NF1-related MPNSTs. *Oncogene.* 2024;43(19):1411–1430.
- Grit JL**, Turner L, Essenburg CJ, et al. Ex vivo patient-derived explant model for neurofibromatosis type 1-related cutaneous neurofibromas. *J Invest Dermatol.* 2024;144(9):2052–2065.e8.
- Benner AD, Rojas FA, Kim SY, **Hou Y**, Coulter KM. COVID-19 Anti-Chinese discrimination, current pandemic stress, and adolescents' mental health. *J Racial Ethn Health Disparities.* 2024;11(4):1946–1955.
- Ip KI, Song J, Wen W, Yan J, **Hou Y**, et al. Unique and interactive roles of economic stress and discrimination on heterogeneous development among Chinese American adolescents: An 8-year longitudinal study. *Dev Psychol.* August 29, 2024. [Epub ahead of print.]
- Kim SY, Song J, Wen W, [...] **Hou Y**. Language brokering profiles of Mexican-origin adolescents in immigrant communities: social-cultural contributors and developmental outcomes. *Child Dev.* 2024;95(4):1237–1253.
- Kim SY, Wen W, Coulter KM, [...] **Hou Y**, et al. Survival analysis and socio-cognitive factors in the timing of COVID-19 vaccination among Mexican-origin youth. *J Racial Ethn Health Disparities.* April 5, 2024. [Epub ahead of print.]



- Kim SY, Wen W, Coulter KM, [...] **Hou Y**, Shen Y. Sociocultural antecedents and mechanisms of COVID-19 vaccine uptake among Mexican-origin youth. *Behav Med*. 2024;1–12.
- Wu N, **Hou Y**, Jiang Y, et al. Longitudinal relations between social relationships and adolescent life satisfaction: the mediating roles of self-compassion and psychological resilience. *J Child Fam Stud*. 2024;33(7):2195–2208.
- Hou Y**, Wu X, Allen T, et al. Longitudinal association between executive function and academic achievement in children with neurofibromatosis type 1 and plexiform neurofibromas. *J Int Neuropsychol Soc*. 2023;29(9):839–849.
- Chang M, Sherief M, **Ioannou M**, et al. NF1 expression profiling in IDH-wildtype glioblastoma: genomic associations and survival outcomes. *Acta Neuropathol Commun*. 2024;12(1):172.
- Ioannou M**, Lalwani K, Ayanlaja AA, et al. MEK inhibition enhances the antitumor effect of radiotherapy in NF1-deficient glioblastoma. *Mol Cancer Ther*. 2024;23(9):1261–1272.
- Schaff LR, **Ioannou M**, Geurts M, et al. State of the art in low-grade glioma management: insights from isocitrate dehydrogenase and beyond. *Am Soc Clin Oncol Educ Book*. 2024;44(3):e431450.
- Kallionpää RA**, Johansson E, Böckerman P, et al. The contribution of morbidity and unemployment for the reduced labor market participation of individuals with neurofibromatosis 1 in Finland. *Eur J Hum Genet*. 2024;32(1):83–90.
- Kallionpää RA**, Peltonen S, Le KM, et al. Characterization of immune cell populations of cutaneous neurofibromas in neurofibromatosis 1. *Lab Invest*. 2024;104(1):100285.
- Loponen N, Ylä-Outinen H, **Kallionpää RA**, et al. Hypertension in NF1: a closer look at the primacy of essential hypertension versus secondary causes. *Mol Genet Genomic Med*. 2024;12(1):e2346.
- Reinhold V, **Kallionpää RA**, Valtanen M, et al. Hospital visits associated with oral infections in patients with neurofibromatosis type 1: a register-based analysis. *Am J Med Genet A*. September 24, 2024. [Epub ahead of print.]
- Keuthan CJ**, Zack DJ. RNA isolation from human stem cell-derived retinal organoids. *Methods Mol Biol*. 2024;2822:3–11.
- Cheng J, **Keuthan CJ**, Esumi N. The many faces of SIRT6 in the retina and retinal pigment epithelium. *Front Cell Dev Biol*. 2023;11:1244765.
- Bhatwadekar AD, **Lukkes (Burke) J**, Mahajan N, et al. Central overexpression of BMAL1 improves circadian rhythm and retinal health in db/db mice. *Invest Ophthalmol Vis Sci*. 2024;65(7):304.
- Chou C, Wiredu SO, Von Imhof L, [...] **Merker VL**. Health services interventions to improve the quality of care in rare diseases: a scoping review. *Ther Adv Rare Dis*. 2024;5:1–13.
- 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*. 2024;21(1):3–5.
- Merker VL**, Thompson HL, Wolters PL, et al. Recommendations for assessing appearance concerns related to plexiform and cutaneous neurofibromas in neurofibromatosis 1 clinical trials. *Clin Trials*. 2024;21(1):6–17.
- Plotkin SR, Yohay KH, Nghiemphu PL, [...] **Merker VL**, et al. Brigatinib in NF2-related schwannomatosis with progressive tumors. *N Engl J Med*. 2024;390(24):2284–2294.
- Silverman D, Da J, **Merker V**, et al. Phase 2 randomized, double-blind, placebo-controlled study of the anti-nerve growth factor (NGF) antibody tanezumab in subjects with moderate to severe pain due to schwannomatosis. *Neurology*. 2024;102(17\_suppl\_1):3692.
- Vogt D, Borowski S, Etingen B, **Merker VL**, et al. Using well-being measurements to enhance clinical practice: why and how to ask patients about their broader well-being. *Med Care*. 2024;62(12 Suppl 1):S73–S75.
- 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*. 2024;21(1):73–84.



- Gerschenfeld G, Couplier F, Gresset A, **Pulh P**, et al. Neural tube-associated boundary caps are a major source of mural cells in the skin. *Elife*. 2023;12:e69413.
- Kucenas S, **Pulh P**, Topilko P, Smith CJ. Glia at transition zones. *Cold Spring Harb Perspect Biol*. June 10, 2024. [Epub ahead of print.]
- Couplier F, **Pulh P**, Oubrou L, et al. Topical delivery of mitogen-activated protein kinase inhibitor binimetinib prevents the development of cutaneous neurofibromas in neurofibromatosis type 1 mutant mice. *Transl Res*. 2023;261:16–27.
- Ahmari N, Perrino M, **Pundavela J**, et al. C5aR-antagonism alone or in combination with MEK inhibition durably alters the plexiform neurofibroma tumor micro-environment. *J Pharmacol Exp Ther*. 2024;389(S3).
- Perrino MR, Ahmari N, Hall A, [...] **Pundavela J**, et al. C5aR plus MEK inhibition durably targets the tumor milieu and reveals tumor cell phagocytosis. *Life Sci Alliance*. 2024;7(5):e202302229.
- Pundavela J**, Dinglasan SA, Touvron M, et al. Stimulator of interferon gene facilitates recruitment of effector CD8 T cells that drive neurofibromatosis type 1 nerve tumor initiation and maintenance. *Sci Adv*. 2024;10(42):eado6342.
- Raut NG**, Maile LA, Oswalt LM, et al. Schwann cells modulate nociception in neurofibromatosis 1. *JCI Insight*. 2024;9(2):e171275.
- Hirbe AC, Dehner CA, Dombi E, [...] **Sundby RT**, et al. Contemporary approach to neurofibromatosis type 1-associated malignant peripheral nerve sheath tumors. *Am Soc Clin Oncol Educ Book*. 2024;44(3):e432242.
- Murphy J, **Sundby RT**, Resch EE, et al. Brain metastasis in pediatric patients with osteosarcoma. *Curr Oncol*. 2024;31(11):7014–7022.
- Sundby RT**, Rhodes SD, Komlodi-Pasztor E, et al. Recommendations for the collection and annotation of biosamples for analysis of biomarkers in neurofibromatosis and schwannomatosis clinical trials. *Clin Trials*. 2024;21(1):40–50.
- Chen J, Pang J, An C, [...] **Zhang L**. Exploring the intersectionality of race and gender on the incidence of and response to microaggression experienced by Asian American women medical students. *Am J Surg*. 2024;239:116007.
- Lee R, Lucas R, Dickerman J, [...] **Zhang L**, Sánchez JP. Designing prefaculty competencies for diverse learners through a modified delphi process. *JAMA Netw Open*. 2024;7(7):e2424003.
- Zhang L**, An C, Chen J, et al. Characterizing Asian American medical students' experiences with microaggression and the impact on their well-being. *Med Educ Online*. 2024;29(1):2299534.
- Liu Z, **Zhang X**, Xu M, et al. MYCN drives oncogenesis by cooperating with the histone methyltransferase G9a and the WDR5 adaptor to orchestrate global gene transcription. *PLoS Biol*. 2024;22(3):e3002240.
- Nguyen R, Doubrovina E, Mousset CM, [...] **Zhang X**, et al. Cooperative armoring of CAR and TCR T cells by T cell-restricted IL15 and IL21 universally enhances solid tumor efficacy. *Clin Cancer Res*. 2024;30(8):1555–1566.
- Xu M, Hong JJ, **Zhang X**, et al. Targeting SWI/SNF ATPases reduces neuroblastoma cell plasticity. *EMBO J*. 2024;43(20):4522–4541.

## Collaborative Publications among NFYIF 2023 Young Investigators

- Sundby RT**, Szymanski JJ, Pan AC, [...] **Murray B**, et al. Early detection of malignant and premalignant peripheral nerve tumors using cell-free DNA fragmentomics. *Clin Cancer Res*. 2024;30(19):4363–4376.
- Wang J**, Calizo A, **Zhang L**, et al. CDK4/6 inhibition enhances SHP2 inhibitor efficacy and is dependent upon RB function in malignant peripheral nerve sheath tumors. *Sci Adv*. 2023;9(47):eadg8876.
- Zhang L**, Maalouf A, Makri SC, [...] **Ioannou M**, et al. Multi-dimensional immunotyping of human NF1-associated peripheral nerve sheath tumors uncovers tumor-associated macrophages as key drivers of immune evasion in the tumor microenvironment. *Clin Cancer Res*. 2024; 30(23):5459–5472.





## Presentations

### *Children's Cancer Foundation (CCF) 8th Annual Research Symposium*

*June 5, 2024; Greenbelt, Maryland*

**Annor GK.** DLK1 is a therapeutic target in MPNST, and its expression can be modulated by epigenetic modifications. Oral Presentation.

### *Children's Tumor Foundation (CTF) European Neurofibromatosis (NF) Group 2024 Global NF Conference*

*June 20–25, 2024; Brussels, Belgium*

**Bhattacharyya S,** Beauchamp RL, Ramesh V. Understanding the role of apelin-mediated angiogenesis in NF2-associated tumors. Poster number 159.

**Grit J,** Turner L, Essenburg C, et al. Targeting inflammatory signaling in cutaneous neurofibromas. Poster number 49.

**Hou Y,** Xiaoli Z, Liy D, et al. Age-varying associations between executive functions and academic achievement in children with neurofibromatosis type 1: integrative analyses of data from seven institutions. Poster number 82.

**Kallionpää R,** Uusitalo E, Leppävirt J, et al. Updated Finnish NF1 cohort: cancer incidence and risk for multiple cancers. Poster number 92.

**Merker V,** Carias S, Murthy N, et al. Providing higher-quality care to people with NF1 who do not attend NF clinics: qualitative interviews with U.S. NF1 patients, parents, and primary care providers. Poster number 130.

**Murray B,** Hong I, **Zhang X,** et al. SWI/SNF ATPase modulation of the DNA damage response in malignant peripheral nerve sheath tumors induces a therapeutic vulnerability in this disease. Poster number 95.

**Pulh P,** Couplier F, Onfroy A, et al. Decipher the mechanisms governing cutaneous neurofibromas development in a mouse model of neurofibromatosis type 1. Poster number 111.

**Pundavela J,** Hall A, Burgard J, et al. Platform: targeting granulocyte-macrophage colony stimulating factor signaling in plexiform neurofibroma. Oral Session Platform Presentation.

**Raut N,** Adlakha A, Sprague KL, et al. GDNF signaling to neurons modulates pain in a preclinical model of NF1. Poster number 119.

**Sundby RT, Zhang X,** Shahsavari S, et al. Circulating protein signatures detect MPNST in patients with NF1. Poster number 145.

**Wang J,** Garcia N, Sarkar A, et al. Platform: vertical inhibition of ERK signaling is effective in preclinical models of malignant peripheral nerve sheath tumors. Oral Session Platform Presentation.

**Zhang L,** Maalouf A, Makri SC, et al. Profiling the immunotypes of the tumor microenvironment in human NF1-associated peripheral nerve sheath tumors. Poster number 157.

**Zhang X,** Abbas S, Syed N, et al. Platform: a comprehensive algorithm to predict malignant transformation of NF1 nerve sheath tumors from single-cell transcriptomic profiling. Oral Session Platform Presentation.

### *2024 Banbury Center Meeting "Cognition and Behavior in Neurofibromatosis Type 1"*

*October 20–23, 2024; Cold Spring Harbor, New York*

**Hou Y.** Using big data to comprehensively delineate the neurobehavioral phenotype of children with neurofibromatosis type 1. Oral Presentation Session 1: Clinical Framework.





### *2024 International Brain Research Organization-Asian Pacific Regional Committee (IBRO-APRC) 4th Advanced School/Neurosociety of Nepal*

*April 24–May 8, 2024; Kathmandu, Nepal*

**Raut, NGR.** The role of Schwann cells in the onset of pain due to neurofibromatosis-1 (NF-1). Platform Presentation.

### *2024 International Association for the Study of Pain (IASP)-World Congress on Pain*

*August 5–9, 2024; Amsterdam, The Netherlands*

**Raut NGR, Adlakha A, Sprague KL, et al.** GDNF signaling from Schwann cells regulates pain in a model of neurofibromatosis 1. Poster number TH397.

## Honors and Awards

### **George Kwakye Annor, PhD**

2024 | The Children's Cancer Foundation (CCF) Research Grant

### **Yang Hou, PhD**

2024–2026 | Florida State University SEED Award

2024–2028 | Department of Defense, Traumatic Brain Injury and Psychological Health Clinical Trial Award

### **Vanessa Merker, PhD**

2024–2025 | Anne Klibanski Visiting Scholar Award at Massachusetts General Hospital

### **Béga Murray, PhD**

2025 | National Institute of Health (NIH) Travel Award: Fellow's Award for Research Excellence (FARE)

### **Namrata Raut, PhD**

2024 | Selected Trainee for North American Pain School (NAPS) – Class of 2024

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

2024 | Children's Tumor Foundation Make NF Visible Research Award

### **Jiawan (Ava) Wang, PhD**

2023–2025 | NIH/NIC Investigator (PI) Role Research Studies (2)

# Start Planning for 2025!



## 5<sup>TH</sup> ANNUAL **NEUROFIBROMATOSIS** Young Investigators' Forum

Abstract submission deadline and forum details coming soon.  
To be notified when the Call for Abstracts is distributed,  
please email [info@ceconcepts.com](mailto:info@ceconcepts.com).

Visit the NFYIF [website](#) for all things NFYIF!

Follow us on  [@NFYIForum](#)

**ceCONCEPTS**   
HEALTHCARE EDUCATION