

2022 PROGRAM NEWSLETTER

2 N D A N N U A L

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

NOVEMBER 2-4, 2022

THE HOUSTONIAN HOTEL | HOUSTON, TEXAS

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Presented by
Creative Educational Concepts, LLC
in collaboration with the
Children's Tumor Foundation.



Supported by an
independent educational
grant from Alexion and
SpringWorks Therapeutics.

Dear Colleagues,

We are all fortunate to be working during a time of such rapid advancement in the field of neurofibromatosis (NF). Despite the challenging and complex nature of NF, the advent of targeted medical therapies, as well as novel diagnostic techniques and a number of other nascent treatment approaches, have recently galvanized much-needed paradigmatic change in a disease historically characterized by limited treatment options, patient morbidity, and bleak prognoses. It is, indeed, an exciting time for patients and clinicians alike. But to sustain and expand this positive momentum, the next generation of NF researchers must continue to be cultivated, mentored, and empowered to build upon existing successes and catalyze NF therapeutics into a new era. The second Neurofibromatosis Young Investigators' Forum (NFYIF) sought to do exactly that, convening high-potential, early-career NF investigators from both across the country and internationally and provisioning a forum in which they could share their work with peers and premier NF thought leaders, who provided expert training and constructive feedback to improve both scientific quality and presentation value. A truly world-class panel of Expert Judge Mentors selected 15 young investigators (clinician scientists, research scientists, clinical fellows, and PhD postdoctoral fellows) based on blinded reviews of submitted abstracts. The invited young investigators presented their work and received feedback from not only the expert panel, but from peers/future collaborators and professional medical communication coaches.

The second annual NFYIF was widely considered a robust success. Young investigator research quality and complexity were illustrative of the very bright future of NF, and poignant sessions led by patients and caregivers personified the empiric "why" behind all that we do. Young investigators rated the meeting highly during post-meeting evaluation, with some calling the NFYIF "a great opportunity to meet future collaborators and career development."

On behalf of the Expert Judge Mentors, we sincerely congratulate the 2022 NFYIF class of young investigators on their fantastic work! We look forward to your future successes and hope the connections and friendships you made at the NFYIF will last a lifetime.

Sincerely,

Bruce R. Korf, MD, PhD (Chair)

Andrea M. Gross, MD (Co-chair)





The Neurofibromatosis Young Investigators’ Forum (NFYIF) is a unique educational opportunity designed for U.S.- and internationally-based young investigators (MD, DO, and/or PhD) 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 judges for assessment.

The 2022 NFYIF—the first in-person initiative—was 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 a robust 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 15 researchers were invited to present their data to peers and an esteemed panel of Expert Judge Mentors in a modified NIH scoring format. In an effort to augment the professional development aspects of this forum, a professional coach 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 was 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 such as NF. Our second annual NFYIF installment robustly achieved this goal, and in so doing, effectively laid the groundwork for future successes.

 **Vanessa L. Merker, PhD**
@vlmerker

#NFYIF22 was an amazing meeting all around, but the best part might just be bonding with this stellar group of fellows and junior faculty committed to #endNF. Signed, the de facto NF social chair 😊




I FOUND MEETING OTHER YOUNG INVESTIGATORS AND DISCUSSING OUR CHALLENGES AND POTENTIAL SOLUTIONS PEER-TO-PEER TO BE A HIGHLIGHT OF THE NEUROFIBROMATOSIS YOUNG INVESTIGATORS’ FORUM.



THE DIVERSITY OF RESEARCH TOPICS PRESENTED BY EACH OF THE YOUNG INVESTIGATORS WAS VERY IMPRESSIVE.



I AM SO ENCOURAGED BY WHAT I SAW AT THE NF YOUNG INVESTIGATORS FORUM... THE IDEAS AND PROJECTS DISCUSSED OFFER A BRIGHT FUTURE FOR NF RESEARCH. BEING IN PERSON AGAIN, I COULD SEE NEW RELATIONSHIPS AND COLLABORATIONS DEVELOP BETWEEN THE YOUNG INVESTIGATORS.

BILL RITER, PATIENT ADVOCATE

**\$5,000
GRANT**

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 Investigators' Award was presented to:

2022 CLINICIAN SCIENTIST AND RESEARCH SCIENTIST

Liyam Laraba, PhD

PLYMOUTH UNIVERSITY
PLYMOUTH, UNITED KINGDOM

Targeting the Hippo Pathway in NF2-null Schwannoma and Meningioma for Drug Screening and Molecular Target Discovery

As a junior faculty research scientist at the University of Plymouth, in Plymouth, England, Dr. Laraba is driven to discover more about the tumour biology of NF2-null schwannomas and meningiomas. He completed his PhD at the University of Plymouth, examining the role of Hippo signaling in schwannoma and meningioma. He then won a 2-year post-doc award from the Children's Tumor Foundation to test promising TEAD inhibitors in preclinical schwannoma models. In 2022, Dr. Laraba was promoted to the role of research fellow and now is assuming supervisory and teaching roles in addition to his research. Currently, he is translating the molecular targets and novel therapies in their preclinical pipeline, to improve clinical outcomes for patients with NF2-related tumours.

“ Participating in the NFYIF has led to an exciting collaboration through networking, as well as given me confidence in presenting. It was also invaluable to meet and chat with experts in the NF field for career guidance.

“ My grant award will be used to fund travel to future international conferences and some lab consumables for a pilot project.



CLINICAL SCIENTIST AND RESEARCH SCIENTIST

1st Runner-Up

Vanessa Merker, PhD

MASSACHUSETTS GENERAL HOSPITAL

Improving Measurement of Quality of Life in NF2 Clinical Trials: Analysis of INTUITT-NF2 Participant Interviews and Patient-reported Outcomes

- “ Attending NFYIF, and especially networking with other young investigators, was an amazing experience. 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 (CTF and Alexion).
- “ I plan to use this award to support undergraduate student(s) working in my lab, allowing me to expose more trainees to NF while also expanding my research assessing NF patients’ preferences and health outcomes.

2nd Runner-Up

Carlos G. Romo, MD

JOHNS HOPKINS UNIVERSITY SCHOOL OF MEDICINE

Natural History Study of Cutaneous Neurofibromas in People with NF1: Results of Phase 1

- “ Participating in the NFYIF was a great experience to learn from other investigators and the mentors and to forge new collaborations.
- “ I will use the grant to create educational material and to cover the costs of publication of research related to NF.



CLINICAL FELLOWS, PHDS AND POSTDOCTORAL FELLOWS

First Place

Russell Taylor Sundby, MD

NATIONAL CANCER INSTITUTE

Dissecting the Circulating Proteome in NF1-associated Peripheral Nerve Sheath Tumors

“ NFYIF affords the opportunity to spend focused time with established leaders in the field and rising stars in NF research. The career and research mentorship that I received has been invaluable and has sparked new projects and collaborations! This type of access and protected brainstorming (both in formal sessions and over drinks in the evening) is impossible at larger conferences where there are meetings and commitments pulling everyone in various directions!

“ The generous NFYIF award will help fund the sequencing and bioinformatic resources required to generate pilot data for novel NF1 biomarkers and techniques.

Second Place

Xiyuan Zhang, PhD

NATIONAL CANCER INSTITUTE

Single-cell Sequencing Reveals Transcriptomic Diversity that Facilitates the Malignant Transformation of NF1 Nerve Sheath Tumors

“ It's my second time participating in the NFYIF and it's, again, a wonderful experience getting to know my fellow YIs. It's extra meaningful because this year we got to meet in person. The friendship and professional network with these wonderful young investigators will benefit my professional development tremendously. I also enjoy learning from the faculty, especially in the format of small group discussions. This is such a unique experience that was made available to us through the NFYIF.

“ I plan to use the grant award to validate the preliminary findings from our single-cell atlas of the NF1-associated nerve sheath tumors. Additional experiments using molecular biology tools become possible because of this award.



CLINICAL FELLOWS, PHDS AND POSTDOCTORAL FELLOWS

Third Place (Tie)

Stephanie Joy Bouley, PhD

MASSACHUSETTS GENERAL HOSPITAL

Targeting the NF- κ B Pathway to Treat NF1-deficient Tumors

“ I am grateful to have attended the NFYIF, particularly at this stage in my career, as it has allowed me to meet others in similar situations and discuss different issues we face as young investigators. Being able to present my work in person to my colleagues was extremely helpful in directing the project’s follow-up work. Finally, having the opportunity to be further integrated into the NF community through networking and potential collaborations developed as a result of the NFYIF has been instrumental in my career development. I am extremely thankful to have received this opportunity.

“ This grant award will be used to continue my research focused on identifying novel therapeutic options to treat NF1-related tumors and to support publishing the work presented at the NFYIF.

Third Place (Tie)

Kyle B. Williams, PhD

MEDICAL COLLEGE OF MINNESOTA

Altered Epigenetic Homeostasis Yields Therapeutic Vulnerabilities in NF1-associated Malignant Peripheral Nerve Sheath Tumors

“ Participating in the NFYIF for the last 2 years has been so valuable in building personal connections with other early career investigators and established faculty within the field. Those interactions and potential collaborations are critical when working in a rare disease space, such as NF.

“ I plan on using the award toward travel costs related to presenting this research at an international conference focused on rare sarcoma biology and therapeutics discovery.





Simge Acar, MD

POSTDOCTORAL YOUNG INVESTIGATOR
WASHINGTON UNIVERSITY-ST. LOUIS

UBR5, a Chr8 Gene, Promotes Tumor Growth of Malignant Peripheral Nerve Sheath Tumors (MPNST)



Jineta Banerjee, PhD

SENIOR SCIENTIST
SAGE BIONETWORKS

Identifying MicroRNA Regulators Facilitating Tumor Progression in Neurofibromatosis Type-1 Using Machine Learning Approaches



Stephanie Joy Bouley, PhD

RESEARCH FELLOW
MASSACHUSETTS GENERAL HOSPITAL

Targeting the NF-kB Pathway to Treat NF1-Deficient Tumors



Maria Loannou, MD

POSTDOCTORAL RESEARCH FELLOW
JOHNS HOPKINS UNIVERSITY

MEK Inhibitors Suppress HR Pathway Expression in NF1-deficient Glioma, Sensitizing Them to Irradiation



Liyam Laraba, PhD

RESEARCH FELLOW
UNIVERSITY OF PLYMOUTH

Targeting the Hippo Pathway in NF2-null Schwannoma and Meningioma for Drug Screening and Molecular Target Discovery

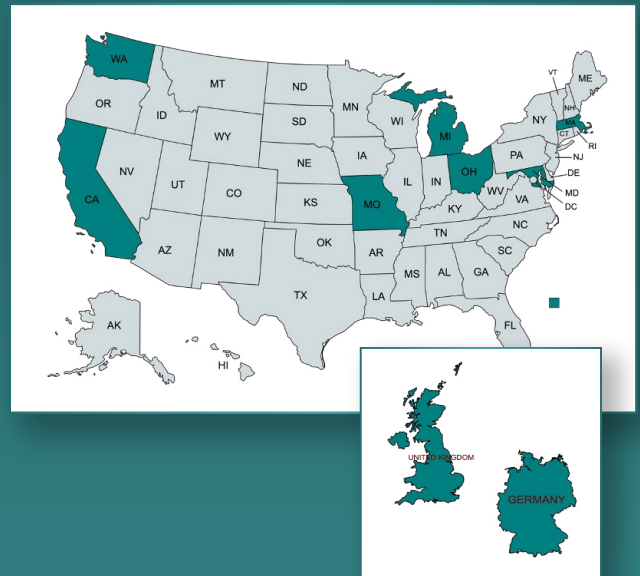


Michelle Mattson-Hoss, PhD

PRINCIPAL SCIENTIST
INFIXION BIOSCIENCE

Identification and Development of a Multifunctional NF1 Monoclonal Antibody

12 INSTITUTIONS
REPRESENTED INTERNATIONALLY



- CINCINNATI CHILDREN'S HOSPITAL
MEDICAL CENTER
- INFIXION BIOSCIENCE
- JOHNS HOPKINS UNIVERSITY SCHOOL
OF MEDICINE
- JOHNS HOPKINS HOSPITAL
- MASSACHUSETTS GENERAL HOSPITAL/
HARVARD MEDICAL SCHOOL
- NATIONAL CANCER INSTITUTE,
PEDIATRIC ONCOLOGY BRANCH
- SAGE BIONETWORKS
- UNIVERSITY MEDICAL CENTER
HAMBURG-EPPENDORF (UKE)
- UNIVERSITY OF MINNESOTA
- UNIVERSITY OF PLYMOUTH
- WASHINGTON UNIVERSITY-ST. LOUIS



Lindy Zhang, MD
@dr_lindy Zhang

Replying to @NFYIForum @rtsundby and 3 others

@rtsundby what an inspiration for younger young investigators! :)

10:36 AM - Nov 4, 2022



Vanessa Merker, PhD
 INSTRUCTOR IN NEUROLOGY
 MASSACHUSETTS GENERAL HOSPITAL/
 HARVARD MEDICAL SCHOOL

Improving Measurement of Quality of Life in NF2 Clinical Trials: Analysis of INTUITT-NF2 Participant Interviews and Patient-reported Outcomes



Jay Pundavela, PhD
 RESEARCH ASSOCIATE
 CINCINNATI CHILDREN'S HOSPITAL MEDICAL CENTER

T cells and Dendritic Cells: Dark Side of the Immune System in Neurofibroma



Namrata G.R. Raut, PhD
 RESEARCH FELLOW
 CINCINNATI CHILDREN'S HOSPITAL MEDICAL CENTER

The Onset of Pain Due to Nf1 is Modulated by Schwann Cell-sensory Neuron Interactions



Inka Ristow, MD
 UNIVERSITY MEDICAL CENTER HAMBURG-EPPENDORF (UKE)

Determination of Diagnostic Cut-off Values for Differentiation of Benign, Atypical, and Malignant Peripheral Nerve Sheath Tumors in NF1 Using Diffusion-weighted Magnetic Resonance Imaging



Carlos G. Romo, MD
 ASSISTANT PROFESSOR OF NEUROLOGY
 JOHNS HOPKINS UNIVERSITY SCHOOL OF MEDICINE

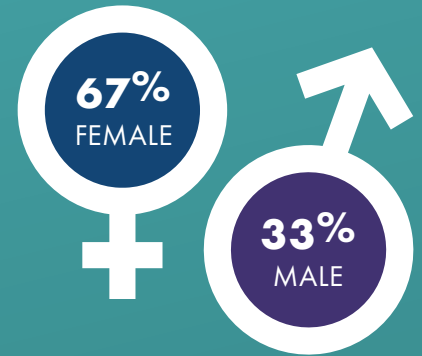
Natural History Study of Cutaneous Neurofibromas in People with NF1: Results of Phase 1



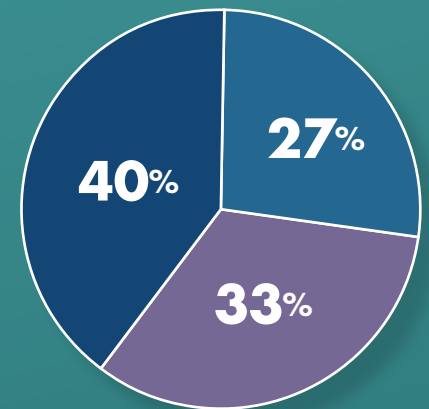
Russell Taylor Sundby, MD
 ASSISTANT RESEARCH PHYSICIAN
 NATIONAL CANCER INSTITUTE,
 PEDIATRIC ONCOLOGY BRANCH

Dissecting the Circulating Proteome in NF1-associated Peripheral Nerve Sheath Tumors

PARTICIPANT DEMOGRAPHICS



PARTICIPANT CATEGORIES



- PhD/Postdoctoral Fellow
- Clinical Fellow
- Jr Faculty



Kyle B. Williams, PhD

POSTDOCTORAL FELLOW
UNIVERSITY OF MINNESOTA

Altered Epigenetic Homeostasis Yields Therapeutic Vulnerabilities in NF1-associated Malignant Peripheral Nerve Sheath Tumors



Lindy Zhang, MD

CLINICAL RESEARCH FELLOW
JOHNS HOPKINS HOSPITAL

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



Xiyuan Zhang, PhD

RESEARCH FELLOW
NATIONAL CANCER INSTITUTE

Single-cell Sequencing Reveals Transcriptomic Diversity that Facilitates the Malignant Transformation of NF1 Nerve Sheath Tumors



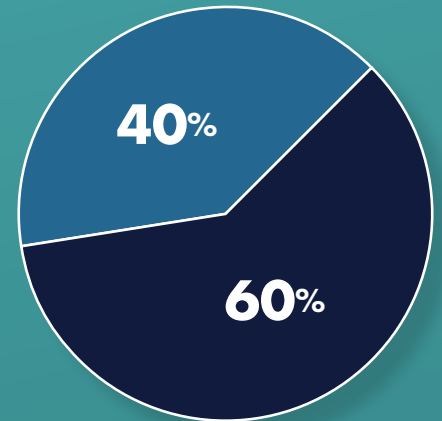
Vanessa L. Merker, PhD
@vlmerker

.@NFYIForum was a blast! I was honored to win first runner-up for junior faculty, and am eagerly watching what the other talented young investigators will achieve for #neurofibromatosis and #schwannomatosis. Thank you to @ChildrensTumor for funding the work I presented! #NFYIF22

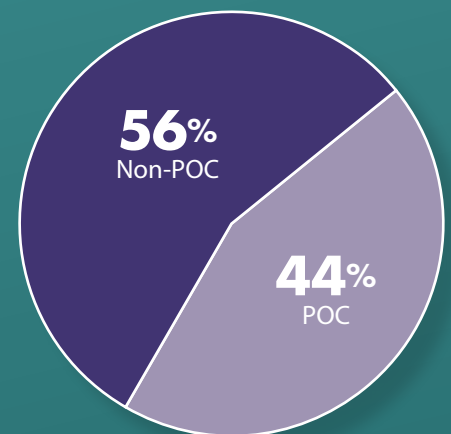


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PARTICIPANT CREDENTIALS



PARTICIPANT DIVERSITY





Bruce R. Korf, MD, PhD

Activity Chair

UNIVERSITY OF ALABAMA
BIRMINGHAM
BIRMINGHAM, AL



IT WAS WONDERFUL TO SPEND SOME TIME AND LEARN ABOUT THEIR PERSONAL STRUGGLES IN RESEARCH AND NF FIELD.



I THOUGHT ALL OF THE MENTORS DID A GREAT JOB AT CHAIRING THE SESSIONS.



Andrea M. Gross, MD

Activity Co-chair

NATIONAL INSTITUTES OF HEALTH
WASHINGTON, DC



Michael J. Fisher, MD

CHILDREN'S HOSPITAL
OF PHILADELPHIA
PHILADELPHIA, PA



AeRang Kim, MD, PhD

CHILDREN'S NATIONAL HOSPITAL
WASHINGTON, DC



Nancy Ratner, PhD

CINCINNATI CHILDREN'S
HOSPITAL MEDICAL CENTER
CINCINNATI, OH

PUBLICATIONS

Acar S, Nieblas-Bedolla E, Armstrong AE, Hirbe AC. A systematic review of recent and ongoing clinical trials in patients with the neurofibromatoses. *Pediatr Neurol.* 2022;134:1–6.

Acar S, Armstrong AE, Hirbe AC. Plexiform neurofibroma: shedding light on the investigational agents in clinical trials. *Expert Opin Investig Drugs.* 2022;31(1):31–40.

Clayton A, DeFelice M, Zalmanek B,...**Banerjee J**, et al. Centralizing neurofibromatosis experimental tool knowledge with the NF Research Tools Database. *Database (Oxford).* 2022;2022:baac045.

Maze EA, Agit B, Reeves S,...**Laraba L**, et al. Human endogenous retrovirus type k promotes proliferation and confers sensitivity to antiretroviral drugs in merlin-negative schwannoma and meningioma. *Cancer Res.* 2022;82(2):235–247.

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.* September 23, 2022. [Epub ahead of print.]

Frost M, Serra E, Viskochil D,...**Mattson-Hoss MK**, et al. Rationale for haploinsufficiency correction therapy in neurofibromatosis type 1. *J Transl Genet Genom.* 2022;6:403–428.

Ly KI, **Merker V**, 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.* November 4, 2022. [Epub ahead of print.]

Da JI, **Merker VL**, Jordan JT, et al. Design of a randomized, placebo-controlled, phase 2 study evaluating the safety and efficacy of tanezumab for treatment of schwannomatosis-related pain. *Contemp Clin Trials.* 2022;121:106900.

Merker VL, Slobogean B, Jordan JT, et al. Understanding barriers to diagnosis in a rare, genetic disease: Delays and errors in diagnosing schwannomatosis. *Am J Med Genet A.* 2022;188(9):2672–2683.

Merker VL, Knight P, Radtke HB, et al. Awareness and agreement with neurofibromatosis care guidelines among U.S. neurofibromatosis specialists. *Orphanet J Rare Dis.* 2022;17(1):44.

Ferdoushi A, Jamaluddin MFB, Li X, **Pundavela J**, et al. Secretome analysis of human schwann cells derived from malignant peripheral nerve sheath tumor. *Proteomics.* 2022;22(1-2):e2100063.

Hornung RS, **Raut NG**, Cantu DJ, et al. Sigma-1 receptors and progesterone metabolizing enzymes in nociceptive sensory neurons of the female rat trigeminal ganglia: a neural substrate for the antinociceptive actions of progesterone. *Mol Pain.* 2022;18:17448069211069255.

Ristow I, Madesta F, Well L, et al. Evaluation of magnetic resonance imaging-based radiomics characteristics for differentiation of benign and malignant peripheral nerve sheath tumors in neurofibromatosis type 1. *Neuro Oncol.* 2022;24(10):1790–1798.

Sundby RT, Pan A, Shern JF. Liquid biopsies in pediatric oncology: opportunities and obstacles. *Curr Opin Pediatr.* 2022;34(1):39–47.

Cortes-Ciriano I, Steele CD, Piculell K,...**Sundby RT**, et al. Genomic patterns of malignant peripheral nerve sheath tumor (MPNST) evolution correlate with clinical outcome and are detectable in cell-free DNA. *Cancer Discov.* 2023:CD-22-0786.

Liu Z, **Zhang X**, Xu M, et al. Loss of CASZ1 tumor suppressor linked to oncogenic subversion of neuroblastoma core regulatory circuitry. *Cell Death Dis.* 2022;13(10):871.

Kershner LJ, Choi K, Wu J, **Zhang X**, et al. Multiple Nf1 Schwann cell populations reprogram the plexiform neurofibroma tumor microenvironment. *JCI Insight.* 2022;7(18):e154513.

Zhang X, Lou HE, Gopalan V, et al. Single-cell sequencing reveals activation of core transcription factors in PRC2-deficient malignant peripheral nerve sheath tumor. *Cell Rep.* 2022;40(12):111363.

PRESENTATIONS

2022 NF Conference

June 18–21, 2022; Philadelphia, Pennsylvania

Banerjee J. Mechanisms of immune escape in NF1-associated peripheral nerve sheath tumors. Poster 7, NF1 Basic Science.

Banerjee J, Brande BM, Scott S, et al. Accelerating exploration of the genomic landscape of neurofibromatosis using harmonized genomic data.

Laraba L, Grimm de Guibert J, Edwards P, et al. TEAD autopalmitylation inhibitors reduce proliferation and drive apoptosis in Merlin-null meningioma and schwannoma both in vitro and in vivo.

Mattson-Hoss M. Developing an ELISA/IHC-capable NF1 monoclonal antibody. Poster 57, NF1 Basic Science.

Mattson-Hoss M, Frost M, Croston G, et al. Developing an ELISA/IHC-capable NF1 monoclonal antibody.

Merker V. Conceptualizing clinical benefit for adults and adolescents with progressive NF2-related tumors: qualitative interview results from the INTUITT-NF2 trial. Poster 152, NF2 Clinical Science.

Merker V, Von Imhof L, Park E, et al. Conceptualizing clinical benefit for adults and adolescents with progressive NF2-related tumors: qualitative interview results from the INTUITT-NF2 trial. Poster 152, NF2 Clinical Science.

Pundavela J, Touvron M, Hummel SA, et al. Intercepting T-cell interaction with conventional dendritic cell subtype 1 (cDC1) precludes plexiform neurofibroma formation.

Raut NGR, Adlakha A, Maile L, et al. Schwann cell-specific deletion of Nf1 leads to pain-related hypersensitivity in mice.

Ristow I. Characterization of benign, atypical and malignant peripheral nerve sheath tumors in patients with neurofibromatosis type 1 using diffusion-weighted magnetic resonance imaging. Poster 104, NF1 Clinical Science.

Ristow I, Apostolova I, Kaul MG, et al. Characterization of benign, atypical, and malignant peripheral nerve sheath tumors in patients with neurofibromatosis type 1 using diffusion-weighted magnetic resonance imaging.

Romo C. Clinical and molecular determinants of outcome for non-optic pathway gliomas in adults with neurofibromatosis type 1. Poster 106, NF1 Clinical Science.

Romo C. Genetic modifiers of cutaneous neurofibroma development in adults with neurofibromatosis type 1: research in progress. Poster 108, NF1 Clinical Science.

Romo C. Multi-parametric biomarker development to predict malignant conversion in patients with neurofibromatosis type 1: research in progress. Poster 110, NF1 Clinical Science.

Romo C. Natural history study of cutaneous neurofibromas in people with NF1: results of phase 1. Poster 112, NF1 Clinical Science.

Romo C, Piotrowski AF, Campian JL, et al. Clinical and molecular determinants of outcome for non-optic pathway gliomas in adults with neurofibromatosis type 1.

Romo C, Patel E, Ramos J, et al. Genetic modifiers of cutaneous neurofibroma development in adults with neurofibromatosis type 1: research in progress.

Romo C, Blakeley JO, Roberts J, et al. Multi-parametric biomarker development to predict malignant conversion in patients with neurofibromatosis type 1: research in progress.

Romo C, Roberts J, Slobogean B, et al. Natural history study of cutaneous neurofibromas in people with NF1: results of phase 1.

Sundby RT. Machine learning integration of multimodal cell-free DNA features enhances detection of peripheral nerve sheath tumors. Poster 124, NF1 Clinical Science.

Sundby RT, Szymanski JJ, Pan AC, et al. Machine learning integration of multimodal cell-free DNA features enhances detection of peripheral nerve sheath tumors.

Williams K. Altered epigenetic homeostasis provides therapeutic vulnerabilities in NF1-associated malignant peripheral nerve sheath tumors. Poster 103, NF1 Basic Science.

Williams K, Larsson A, Tibbits J, et al. Altered epigenetic homeostasis provides therapeutic vulnerabilities in NF1-associated malignant peripheral nerve sheath tumors.

Zhang L, Pollard K, Calizo A, Banerjee J, et al. Mechanisms of immune escape in NF1-associated peripheral nerve sheath tumors.

PRESENTATIONS

2022 Society for Neuro-Oncology Annual Scientific Meeting and Education Day

November 16–20, 2022; Tampa, Florida

Romo C, Ellingson B, Strowd R, et al. Determining the dose of regadenoson most likely to transiently alter the integrity of the blood-brain barrier in patients with gliomas. Abstract DDEL-11.

25th American Society of Gene & Cell Therapy (ASGCT) Annual Meeting

May 16–19, 2022; Washington, DC

Bouley SJ, Fernandez F, Scullion EJM, et al. Correcting pathogenic germline mutations in neurofibromatosis type 1 (NF1) using novel gene therapies. Abstract 1045.

American Association for Cancer Research Annual Meeting

April 8–13, 2022; New Orleans, Louisiana

Williams KB, Larsson Z, Tibbits J, et al. Creation of malignant peripheral nerve sheath tumor models deficient for polycomb repressive complex 2 and identification of therapeutic vulnerabilities. Abstract 1614.

Zhang X, Lou HE, Gopalan V, et al. Loss of PRC2 enforces a mesenchymal neural crest stem cell phenotype in NF1-deficient cancer through activation of core transcription factors. Abstract 701.

AWARDS

Laraba L. Dr. Laraba was awarded funding to recruit for a PhD studentship, financially supported by University of Plymouth School of Medicine and Vivace Therapeutics Inc.

Mattson-Hoss MK. Dr. Mattson-Hoss was awarded a grant for “Steric-blocking antisense oligonucleotide (ASO) discovery to selectively correct NF1 haploinsufficiency: R43 (SBIR phase 1).”

Merker V. The Department of Defense Neurofibromatosis Research Program recommended Dr. Merker’s application, “Implementing an online platform to promote evidence-based care for children and adults with neurofibromatosis 1,” for funding under the FY22 New Investigator Award program. She also recently received funding from NF Northeast and the Neurofibromatosis Therapeutic Acceleration Program for research related to patient-reported outcomes and qualitative interviews in NF clinical trials. Dr. Merker was also appointed as a section editor at *Orphanet Journal of Rare Diseases* for the Registries, Health Planning, Health Services, and Quality of Life section.

Sundby RT. Dr. Sundby received the 2022 Francis S. Collins Scholar award, receiving 3 years of salary support, research funding, and educational resources to focus on NF1 research. He also was awarded 2nd Place Clinical Science Poster at the Children’s Tumor Foundation (CTF) Annual NF1 conference. Further, Dr. Sundby was promoted to assistant research physician, joining the NCI Pediatric Oncology Branch faculty.

Zhang X. Dr. Zhang received the Crystal Mackall Excellence in Research Award, NCI POB Research Roundup; the Concept Award in Rare Cancer Research, U.S. Department of Defense; the Scholar in Training Award, American Association for Cancer Research; and the Outstanding Postdoctoral Fellow Award, National Cancer Institute.

CTF MISSION MOMENT: PATIENT AND CAREGIVER PERSPECTIVES

The 2nd annual 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 and internationally; the result was the presentation of highly 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,” in hearing Bill Riter’s story.

The CTF Mission Moment gave the stage to two patients, Bill Riter, who was able to attend in person, and Eddie Purtell, who provided an impact statement of how NF research has impacted his life, alongside advocate Kate Kelts, RN, BSN, who moderated. During the CTF Mission Moment, both Bill and Eddie shared their personal journeys with neurofibromatosis. They inspired, informed, and actuated everyone in the room, continuing to humanize the science and leaving us all in awe of their strength, courage, and determination. The impact Bill and Eddie had on the NFYIF was both tangible and intangible and we very much hope to have them and many other patients and caregivers share their stories at future installments of the NFYIF.



Bill Riter
PATIENT ADVOCATE
CLEVELAND, OHIO



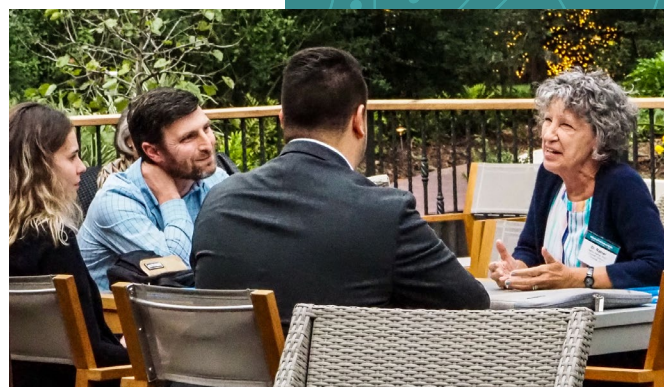
Eddie Purtell
PATIENT ADVOCATE
DENVER, COLORADO



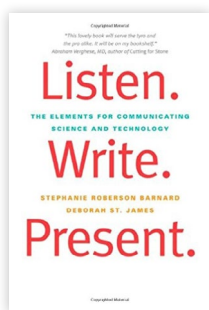
Kate Kelts, RN, BSN
CTF PATIENT SUPPORT ADVOCATE
CHAMPAIGN, ILLINOIS

MENTORING MOMENTS AND MEET-THE-PROFESSOR LUNCH

Guided by the overarching mission to cultivate, inform, and empower young neurofibromatosis researchers, the 2nd annual NFYIF provided an intimate setting in which our young investigators were privy to small group sessions as well as a Mentoring Moment Session with our Expert Judge Mentors. The Expert Judge Mentors provided our young investigators with actionable, real-world advice on circumventing the prominent obstacles faced during early-career inflection points. During the Mentoring Moments session, the Expert Judge Mentors shared practical pearls for navigating the drug development landscape within NF, such as the impact of COVID, shortage of PhD post-docs within the field and beyond. The Expert Judge Mentors also touched upon the challenges seen within clinical trial design and implementation, such as diversity within clinical trial types, data sharing and open science with the NF community, and patient selection. The young investigators were able to pose questions to the panel of NF thought leaders as well as to their peers, creating an open discussion amongst the group as a whole. The Small Group Breakout Discussion session and Meet-the-Professor Lunch provided the YIs and Expert Judge Mentors with a protected and uninterrupted space to connect, provide mentorship, exchange ideas. Within the 2nd Annual NFYIF, there was a great mixture of returning and new Young Investigators, as well as international Young Investigators, providing varying perspectives within this rare disease, and thus further connecting the unconnected.



PRESENTATION SKILLS ENHANCEMENT WORKSHOP



To augment the professional development aspects of the NFYIF, 2022 participants participated in 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 effectively answering questions about and defending their research.

2022 NFYIF Professional Scientific Communication Coach

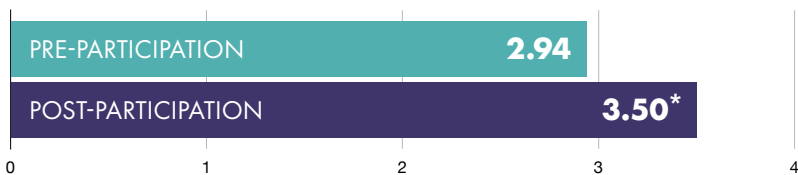


Stephanie Roberson Barnard (Coach)

LISTEN WRITE PRESENT, LLC
GREENSBORO, NC

Change in Confidence in Ability to Defend Research

As a result of attending this activity, participants are better able to improve public speaking and presentation skills to effectively communicate research findings to the larger scientific community.



Mean Confidence on a scale of 1 to 4, where 1 is "not confident" and 4 is "extremely confident" *p=0.0014

2022 NFYIF attendees found the ListenWritePresent Profession Skills Coaching Workshop to be an extremely valuable component of the NFYIF curriculum, ranking the coaching sessions with a mean score of

3.75 out of 4.0

HOW ATTENDING THE NFYIF WILL IMPACT YOUNG INVESTIGATORS' CAREERS



“ I HAVE HAD GREAT DISCUSSIONS FOLLOWING NETWORKING WITH OTHER YOUNG INVESTIGATORS AND WE WILL BE IN CONTACT TO DISCUSS POTENTIAL COLLABORATION IDEAS.

“ THE MEETING...HAS PROVIDED ME WITH EXCELLENT NETWORKING OPPORTUNITIES TO LIKE-MINDED NF RESEARCHERS AT A SIMILAR CAREER STAGE.

“ INTERACTION WITH MENTORS PROVIDED VALUABLE INFORMATION REGARDING APPLYING FOR GRANTS AND PUBLICATIONS.



3 R D A N N U A L
NFYIF

SUMMER/FALL 2023
THE HOUSTONIAN HOTEL
HOUSTON, TEXAS

Keep watch for future announcements on the third annual NFYIF
in the coming months on Twitter @**NFYIForum**.