

**PROGRAM BOOK**

**CHILDREN'S  
TUMOR  
FOUNDATION**  
ENDING NF  
THROUGH RESEARCH

**2023**  
**NF CONFERENCE**  
**JUNE 24-27, 2023**  
FAIRMONT SCOTTSDALE PRINCESS  
ARIZONA

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Network: nfconference  
Password: endnf23

Dear NF Conference Attendees:

On behalf of the Children's Tumor Foundation, I welcome you to the 2023 NF Conference in sunny Scottsdale, Arizona.

It is an honor and inspiration to assemble the entire NF community: NF scientists, clinicians, volunteers, donors, families, patients, and CTF staff—together, we form a mighty front against NF! I truly hope you enjoy this opportunity to meet old friends, forge new connections, share knowledge, celebrate each other's successes, and continue to strengthen and grow this community passionately working to end NF.

Our dedicated NF Conference Chairs (Piotr Topilko, PhD from INSERM, France, Verena Staedtke, MD, PhD from Johns Hopkins University, and Cristina Fernandez-Valle, PhD from the University of Central Florida), alongside CTF's dedicated Conference planners, Patrice Pancza and Angela Dumadag, have planned an exciting agenda with an impressive list of keynote speakers:

- Frank McCormick, PhD, FRSC, from University of California San Francisco will open the Conference with a discussion surrounding Drug Discovery
- Ross Cagan, PhD, from University of Glasgow will talk about RAS Pathways
- Ivana Trapani, MD, PhD, from Telethon Instit of Gen and Medicine, Naples will cover gene engineering for Rare Disease
- Min Jae (MJ) Song, Senior Scientist at the NIH's National Center for Advancing Translational Sciences will speak about Novel Alternative Models (NAMs)
- Michelle Monje, MD, PhD from Stanford University, will give her talk about neuronal regulation contributions to NF1-related tumors.

In addition to our keynotes, we've planned an array of sessions dedicated to preclinical methodologies, RASopathy, and gene therapy, to name a few, as well as a panel discussing Pain and Itch, from basic science all the way to the clinic. On Monday, we've dedicated an entire day to NF2.

I wish you an informative, inspiring, and enjoyable Conference experience. This annual event is a testament to what we can accomplish when we share knowledge and foster collaboration. Our impact shows in the remarkable advancements in care and treatments, as we grow ever closer to ending NF.



**Annette Bakker, PhD, President**

Children's Tumor Foundation

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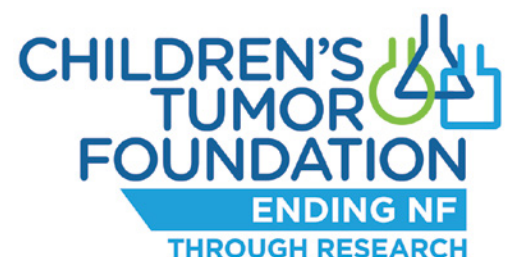
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\*up to date through June 1, 2023



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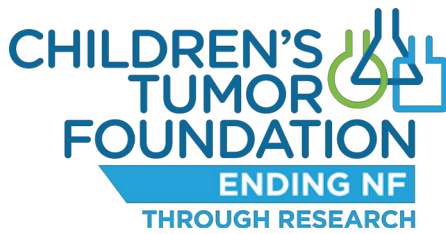
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\*up to date through June 1, 2023







The Children's Tumor Foundation (CTF) offers various grant programs to both academic groups and industries to advance neurofibromatosis and schwannomatosis research

[ctf.org/funding-opportunities](https://ctf.org/funding-opportunities)

#### **YOUNG INVESTIGATOR AWARD (YIA)**

The Young Investigator Award (YIA) provides up to three years of salary support to graduate students and postdoctoral investigators to conduct NF research under the guidance of an established mentor, with the goal of enabling these early career researchers to become established as independent NF investigators. Applications are selected not only for their scientific merit but also for the potential and commitment of the applicant to pursue a career as an independent NF researcher.

#### **DRUG DISCOVERY INITIATIVE (DDI)**

The goal of the Drug Discovery Initiative is to stimulate NF drug discovery by funding researchers proposing to investigate novel or repurposed therapies for NF or to develop tools that support such research. Proposals are expected to be short and concentrated on obtaining key preliminary data needed to quickly advance to the next step of drug discovery.

#### **CLINICAL RESEARCH AWARD (CRA)**

The Clinical Research Award supports early-stage pilot clinical trials of candidate therapeutics or interventions for treatment of NF. This grant mechanism also supports adjunct studies such as biomarker development, imaging protocols, and other clinical trial tools.

#### **CONTRACT AWARD (CA)**

The Contract Awards (CA) are 'by-invitation only' special awards established to fund research that is outside the scope of the above programs. The funding amount and duration depend on the nature of the proposed study.

For Pharma/Biotech companies: If you have any products that could have applicability in NF, we would like to hear from you. CTF is actively seeking opportunities to collaborate with companies interested in developing assets for neurofibromatosis or schwannomatosis. Please contact [abakker@ctf.org](mailto:abakker@ctf.org).

**2024 Grant Cycles will be announced in late Summer 2023.**  
**For updates and more information please visit: [ctf.org/funding-opportunities](https://ctf.org/funding-opportunities)**

**Children's Tumor Foundation Europe is offering travel grants to a selection of multidisciplinary NF centres throughout Europe, to further the professional development of clinicians and allied healthcare professionals who see NF patients. Participating clinics offer a pre-programmed 1-to 3-day visit, as well as à la carte training.**

- Erasmus Medical Center, Rotterdam, Netherlands
- Hôpital Pitié-Salpêtrière, Paris, France
- Universitätsklinikum Hamburg-Eppendorf, Hamburg, Germany
- Guy's and St Thomas' Hospital, London, UK
- Hôpital Henri-Mondor, Créteil, France (greater Paris area)
- UZLeuven, Leuven, Belgium
- Medical University, Vienna, Austria

### **To Apply**

Applicants are invited to submit a CV along with an explanation of their motivation for applying and a description of the program or à la carte training they wish to receive. Applications should be submitted at least 3 months in advance of the time frame in which you hope to attend, to the clinic where you wish to be trained.

### **Grant Amounts and Reimbursement**

Once selected by the clinic and the program visit has been planned, the grantee will be contacted by CTF with a reimbursement procedure. CTF Europe will reimburse up to 500 Euro for travel in economy class, up to 150 Euros per night for lodging, and up to 50 Euros per day for food.

If you have questions, please reach out to the contact at the NF center to which you are interested in applying. For general questions, please reach out to CTF Europe Scientific Officer Marco Nievo at [mnievo@ctf.org](mailto:mnievo@ctf.org)

For more information please visit:  
**[ctfeurope.org/research](https://ctfeurope.org/research)**



# MASTERCLASSES IN **NF**

A series of online educational lectures for medical professionals by leading neurofibromatosis experts

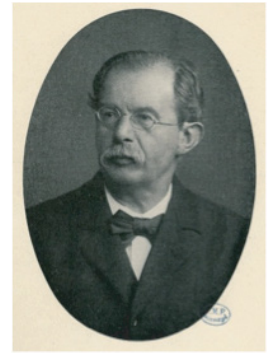
Reserve your spot today:  
[ctfeurope.org/nfmasterclasses](https://ctfeurope.org/nfmasterclasses)

Supported by an educational grant from AstraZeneca



**INFER**  
INTERNATIONAL  
NF  
EDUCATIONAL  
RESOURCES

# The Friedrich von Recklinghausen Award: NF Tradition and Progress



The Children's Tumor Foundation's Friedrich von Recklinghausen Award is given to individuals in the professional NF community who have made significant contributions to neurofibromatosis or schwannomatosis research or clinical care. It is named after Friedrich Daniel von Recklinghausen (1833-1910), the German physician who first described 'von Recklinghausen's disease'—what we now know as neurofibromatosis type 1.



## 2023 Friedrich von Recklinghausen Award Recipient

It is with great pleasure that the Children's Tumor Foundation announces the recipient of the 2023 Friedrich von Recklinghausen Award, Margaret Wallace, PhD.

Dr. Margaret Wallace (aka Peggy) is a cornerstone NF field researcher. While she was a postdoctoral Young Investigators Award (YIA) fellow in the lab of Dr. Francis Collins at the University of Michigan, she played a vital role in cloning the *NF1* gene in 1990. After her YIA fellowship, Peggy established her molecular genetics lab at the University of Florida's College of Medicine, and has since focused on finding and characterizing gene variants/mechanisms that contribute to NF manifestations through approaches of molecular genetics/genomics, cell biology, and animal models.

Peggy has numerous groundbreaking research papers and is the prime example of teamwork and open sharing. She created the first openly available NF immortalized cell lines that shipped these valuable research tools to researchers all over the world.

Together with Dr. Andrea McClatchey, she co-chaired the highly successful Young Investigator Award program for over 15 years, which has funded nearly 200 young researchers leading to a wealth of new insights, hundreds of publications, follow-up funding for NF from other agencies, new researchers in the field, preclinical studies and clinical trials.

Since 2018, Peggy also leads a group of world-class experts in NF1 gene therapy that focuses on gene delivery and gene editing.

The Children's Tumor Foundation, along with her colleagues and peers, is proud and thrilled to recognize Dr. Wallace with the 2023 Friedrich von Recklinghausen Award, not only for her many outstanding achievements over her years in the field but also for her dedicated efforts in supporting the entire NF community. Please join us in congratulating Dr. Wallace for this well-deserved honor.

The following are the most recent recipients of the Award:



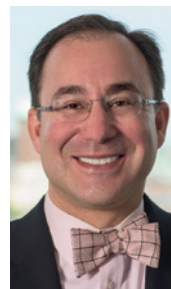
**2022**  
Jaishri Blakeley, MD  
Johns Hopkins University



**2021**  
Marco Giovannini, MD, PhD  
UCLA



**2020**  
D. Wade Clapp, MD  
Indiana University  
School of Medicine



**2019**  
Scott Plotkin, MD, PhD  
Massachusetts General Hospital  
Harvard Medical School

**2014**  
Gareth Evans, MD  
St. Mary's Hospital,  
University of Manchester, UK

**2013**  
Brigitte Widemann, MD  
National Cancer Institute

**2012**  
David Gutmann, MD, PhD  
Washington University

**2010**  
Nancy Ratner, PhD  
Cincinnati Children's Hospital  
Medical Center

**2009**  
Luis Parada, PhD  
University of Texas  
Southwestern

**2008**  
Vincent 'Vic' Riccardi, MD  
The Neurofibromatosis Institute



**2018**  
Ludwine Messiaen, PhD  
University of Alabama  
at Birmingham



**2017**  
Karen Cichowski, PhD  
Harvard Medical School



**2016**  
David Viskochil, MD, PhD  
University of Utah



**2015**  
Eric Legius, MD, PhD  
University of Leuven, Belgium

# SCHEDULE

## Schedule At-A-Glance (All times are MST)

	TIME	EVENT	LOCATION
FRI 6/23	1:30 PM - 5:30 PM	REINS Meeting – Non-CME Activity	Palomino 4-5
	3:00 PM - 8:00 PM	Check In & Registration Opens	Princess South Foyer
	6:00 PM - 7:30 PM	NFCN Meeting & Dinner – By Invitation Only – Non-CME Activity	Fairmont Gold Meeting A
SATURDAY JUNE 24	7:00 AM - 5:00 PM	Check In & Registration	Princess South Foyer
	8:00 AM - 11:30 AM	<b>CLINICAL CARE SYMPOSIUM</b>	Salons D-G
	11:40 AM - 12:00 PM	<b>OPENING REMARKS &amp; MISSION MOMENT</b>	Salons D-G
	12:00 PM - 12:30 PM	Break	Salons A-C
	12:30 PM - 1:30 PM	<b>KEYNOTE #1: Drug Discovery</b>	Salons D-G
	1:30 PM - 2:00 PM	Break	Salons A-C
	2:00 PM - 3:15 PM	<b>SESSION 1: Tips and Tricks for Successful Preclinical Drug Assays</b>	Salons D-G
	3:30 PM - 5:15 PM	<b>SESSION 2: Pain &amp; Itch</b>	Salons D-G
	5:15 PM - 6:15 PM	Networking – Non-CME Activity	Salons A-C
	6:15 PM - 6:45 PM	Special Poster Session & Cocktail Reception – Non-CME Activity	Princess East & North Foyer
7:00 PM - 9:00 PM	<b>Combined NF Conference – NF Summit Welcome &amp; Awards Dinner – Non-CME Activity</b>	Palomino 1-5	
SUNDAY JUNE 25	7:00 AM - 8:00 AM	VCEP Symposium – Non-CME Activity	Salons D-G
	7:00 AM - 9:00 AM	Sunday Breakfast	Salons A-C
	7:00 AM - 3:00 PM	Check In & Registration	Princess South Foyer
	8:30 AM - 9:30 AM	<b>KEYNOTE #2: RAS Pathways</b>	Salons D-G
	9:30 AM - 10:35 AM	<b>SESSION 3, PART 1: Rasopathies</b>	Salons D-G
	10:35 AM - 11:00 AM	Break	Salons A-C
	11:00 AM - 12:00 PM	<b>SESSION 3, PART 2: Rasopathies</b>	Salons D-G
	12:00 PM - 1:00 PM	Sunday Lunch	Salons A-C
	1:00 PM - 2:00 PM	<b>KEYNOTE #3: Gene Engineering for Rare Disease</b>	Salons D-G
	2:00 PM - 3:30 PM	<b>SESSION 4A, PART 1: Preclinical Development: How to Create a Path for Gene Therapy in NF (Concurrent Session)</b>	Salons D-G
	2:00 PM - 3:30 PM	<b>SESSION 4B, PART 1: NF1 Clinical (Concurrent Session)</b>	Salons H-I
	3:30 PM - 3:45 PM	Break	Salons A-C
	3:45 PM - 4:45 PM	<b>SESSION 4B, PART 2: NF1 Clinical (Concurrent Session)</b>	Salons H-I
	3:45 PM - 5:00 PM	<b>SESSION 4A, PART 2: Clinical Development: How to Create a Path for Gene Therapy in NF (Concurrent Session)</b>	Salons D-G
	5:00 PM - 5:15 PM	Poster Overview	Salons D-G
	5:30 PM - 7:30 PM	Poster Session and Cocktail Reception – Non-CME Activity	Princess East & North Foyer
	MONDAY JUNE 26	7:00 AM - 1:00 PM	Check In & Registration
7:00 AM - 8:30 AM		Independent Satellite Symposium – Non-CME Activity	Salons H-I
7:00 AM - 9:00 AM		Monday Breakfast	Salons A-C
8:30 AM - 9:30 AM		<b>KEYNOTE #4: Novel Alternative Models (NAMs)</b>	Salons D-G
9:30 AM - 10:30 AM		<b>SESSION 5A, PART 1: Novel Models (Concurrent Session)</b>	Salons D-G
9:30 AM - 10:30 AM		<b>SESSION 5B, PART 1: NF2/SWN Clinical (Concurrent Session)</b>	Salons H-I
10:30 AM - 11:00 AM		Break	Salons A-C
11:00 AM - 12:00 PM		<b>SESSION 5A, PART 2: Novel Targets (Concurrent Session)</b>	Salons D-G
11:00 AM - 12:00 PM		<b>SESSION 5B, PART 2: NF2/SWN Clinical (Concurrent Session)</b>	Salons H-I
12:00 PM - 12:45 PM		Monday Lunch	Salons A-C
12:45 PM - 1:45 PM		<b>KEYNOTE #5: Neuron-Glial Interactions in NF1: Implications for Cognition and Tumorigenesis</b>	Salons D-G
1:45 PM - 2:45 PM		<b>SESSION 6, PART 1: Aligning Preclinical Studies with Clinical Trial Outcome Measures</b>	Salons D-G
2:45 PM - 3:15 PM		Break	Salons A-C
3:15 PM - 4:45 PM		<b>SESSION 6, PART 2: Aligning Preclinical Studies with Clinical Trial Outcome Measures</b>	Salons D-G
4:45 PM - 5:00 PM		Break	Salons A-C
5:00 PM - 7:00 PM		<b>SESSION 7B: Platform Session – Basic Science (Concurrent Session)</b>	Salons H-I
5:00 PM - 7:00 PM		<b>SESSION 7A: Platform Session – Clinical Science (Concurrent Session)</b>	Salons D-G
TUESDAY JUNE 27	7:00 AM - 9:00 AM	Tuesday Breakfast	Salons A-C
	8:30 AM - 9:30 AM	<b>SESSION 8: Poster Competition – Oral Presentations</b>	Salons D-G
	9:30 AM - 11:00 AM	<b>SESSION 9A: Late Breaking Abstracts (Concurrent Session)</b>	Salons D-G
	9:00 AM - 11:00 AM	<b>SESSION 9B: Industry Platforms (Concurrent Session) – Non-CME Activity</b>	Salons H-I
	11:00 AM - 11:15 AM	Break	Salons A-C
	11:15 AM - 1:15 PM	Consortia and Collaboration Updates	Salons D-G
	1:15 PM - 1:30 PM	<b>FAREWELL AND ADJOURNMENT</b>	Salons D-G



**Cristina Fernandez-Valle, PhD**, *University of Central Florida, College of Medicine*

Cristina Fernandez-Valle, Ph.D. is a Pegasus professor at the University of Central Florida, College of Medicine. She received her Ph.D. from the University of Miami Miller School of Medicine and conducted post-doctoral research there on mechanisms regulating Schwann cell myelination. Over the last 25 years, her research has focused on merlin tumor biology and more recently on identifying therapeutics for NF2-associated schwannomas and pain mechanisms in schwannomatosis.



**Verena Staedtke, MD, PhD**, *Johns Hopkins School of Medicine*

Verena Staedtke, Associate Professor of Neurology, is a physician-scientist and pediatric neuro-oncologist at Johns Hopkins University with a clinical practice devoted to caring for children and young adults with neurofibromatosis 1 & 2 and related conditions. She completed a pediatric neurology residency, a neuro-oncology fellowship and a postdoctoral fellowship in experimental therapeutics at Johns Hopkins. She has built an extensive research expertise in the development of novel immunotherapeutic approaches for brain tumors and cancer animal models. Her current work focuses on the development of gene therapy approaches in NF1 combining the use of innovative vector engineering platforms with payload development. Her work has been supported by several prestigious awards including the Francis S. Collins Scholar Award, the Sontag

Distinguished Scientist Award as well as NCI/NIH K08, NCI/NIH U01 and NTAP.



**Piotr Topilko, PhD**, *Mondor Institute for Biomedical Research, Creteil, France*

Piotr Topilko, PhD is PU-PH (University Professor-Hospital Practitioner) of Histology and Embryology and head of the group “Neurofibromatosis type 1”, that is part of Team 9 “Neurofibromatosis and lymphoma oncogenesis”, at Mondor Institute for Biomedical Research at Creteil, France. Until 2019 he was appointed Research Director at INSERM and head of the group “Development of peripheral nervous system” at Institute of Biology (IBENS) of the Ecole Normale Supérieure in Paris. He is leading outstanding research on Schwann cells, neural crest derived boundary cap (BCs) cells and more recently neurofibromatosis type 1. At present, his major research topics focus on mechanisms governing development of neurofibromas, nerve sheath tumors developing in Neurofibromatosis type 1 (NF1) patients and their progression into

malignancy. He has published 80 publications in high-ranking international journals including first or last author positions in Cell, Nature, Nature Neuroscience, Neuron, Journal of Neuroscience, Development, Cell Stem Reports and Cancer Discovery. Over the last decade he has been actively involved in training neuro- and developmental biology fellows, graduate and medical students.

## 2023 NF Conference Keynote Speakers



**Ross Cagan, PhD**, *University of Glasgow*

Dr. Cagan received his Ph.D. from Princeton University. After a postdoctoral fellowship at UCLA, he achieved the rank of Professor at Washington University School of Medicine (1993-2007), then at Icahn School of Medicine at Mount Sinai (2007-2020). He is currently Regius Professor of Precision Medicine and Scientific Director of the Wolfson Wohl Cancer Research Centre at University of Glasgow, and a Wohl Fellow of the Royal Society. Dr. Cagan is founder and Director of the McNab Centre for Cancer Innovation and Academic Lead for UKRI's SIFP Living Laboratory to help develop Glasgow's health and biotechnology future. He was co-Founder of the biotechnology company Medros Inc. and Board member of the Drosophila company Vivan, the latter built on his laboratory's technologies.

Dr. Cagan is an expert in utilizing *Drosophila* to explore cell-cell signaling and epithelial patterning. Trained as a geneticist and developmental biology, in recent years Dr. Cagan has focused on translational science. He is a pioneer in the use of *Drosophila* to identify therapeutic leads for cancer (breast, lung, thyroid, colorectal), inherited Rasopathies including NF1 and Noonan. Taking advantage of a century of powerful genetic tools, his laboratory has developed complex, multigenic disease models designed to model aspects of the whole-body complexity of human disease. This approach helped validate the first FDA-approved chemotherapeutic for Medullary Thyroid Carcinoma; additional leads are being pursued for cancer and RASopathies.

Working with chemist Arvin Dar and colleagues, his laboratory has developed a novel platform that combines genetics with medicinal and computational chemistry to build novel lead compounds that emphasize rational polypharmacology; his group now works with Lee Cronin and colleagues to merge these efforts with Chemputer technology to fully automate the process. Leveraging these new technologies, Dr. Cagan led the Center for Personalized Cancer Therapeutics team that developed a personalized fly-to-bedside, open label clinical trial for thyroid and cancer patients. His current efforts include guiding integration of new technologies to identify treatments for RAS-dependent disease.

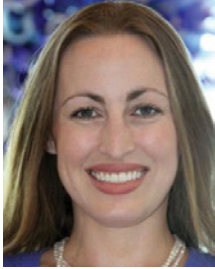
Regarding education, Dr. Cagan served as Senior Associate Dean of the PhD programme at Mount Sinai, developing a series of innovative approaches to prepare students for modern day science careers. Through collaborations and NGOs such as DrosAfrica, he is working with local communities to mentor a new generation of scientists in translational approaches to therapeutics, locally as well as in America, Europe, and Africa.



**Frank McCormick, PhD, FRS, DSc**, *University of California, San Francisco*

Dr. McCormick is a prominent expert in drug discovery in the field of RAS pathways.

## 2023 NF Conference Keynote Speakers



**Michelle Monje, MD, PhD**, *Stanford University*

Dr. Monje is a prominent expert in molecular and cellular mechanisms of postnatal neurodevelopment including microenvironmental influences on neural precursor cell fate choices.

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**Min Jae Song, PhD**, *National Center for Advancing Translational Sciences*

Dr. Song is an expert in the development of Novel Alternative Models (NAMs) and 3D models in a high throughput platform for preclinical studies.

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**Ivana Trapani, MD, PhD**, *TIGEM*

Dr. Trapani is a prominent expert in developing AAV-based gene replacement and genome editing approaches, which overcome the limitations of the existing methodologies.



# AGENDA

## Friday · June 23, 2023 (All times are MST)

1:30 PM	5:30 PM	<b>REiNS MEETING – Non-CME Activity</b>	Palomino 4-5
		Optional Satellite  THE REiNS International Collaboration is a volunteer group of researchers, clinicians, and patients/family members who work to improve the design of NF clinical trials. At this meeting, you can hear about recent REiNS efforts and recommendations for clinical trial endpoints; learn about different forums to interact with the FDA regarding clinical trial innovations; and give input on an upcoming natural history study on NF1 bone issues. Meeting open to all.	
3:00 PM	8:00 PM	<b>CHECK IN &amp; REGISTRATION</b>	Princess South Foyer
6:00 PM	7:30 PM	<b>NFCN MEETING – BY INVITATION ONLY – Non-CME Activity</b>	Fairmont Gold Meeting A

## Saturday · June 24, 2023 (All times are MST)

7:00 AM	5:00 PM	<b>CHECK IN &amp; REGISTRATION</b>	Princess South Foyer
8:00 AM	11:30 AM	<b>CLINICAL CARE SYMPOSIUM</b>	Salons D-G
		Session Co-Chairs: Nicole Ullrich, MD, PhD, <i>Boston Children's Hospital</i> ; Heather Radtke, MS, CGC, <i>Children's Tumor Foundation</i>	
8:00 AM	8:20 AM	<b>Welcome:</b> Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital</i> ; Amedeo Azizi, MD, <i>Medical University of Vienna</i>	
8:20 AM	8:50 AM	<b>Mosaicism in Neurofibromatosis and Schwannomatosis – Clinical Manifestations and Implications for Testing</b> Miriam Bornhorst, MD, <i>Children's National Hospital</i>	
8:50 AM	9:20 AM	<b>Diagnostic Odyssey in Schwannomatosis: How Can We Improve Diagnostic Delays, Errors, and Communication?</b> Vanessa L. Merker, PhD, <i>Massachusetts General Hospital</i>	
9:20 AM	9:40 AM	<b>BREAK</b>	
9:40 AM	10:10 AM	<b>Optic Pathway Glioma and MRI: To Screen or Not to Screen? That is the Question</b> Rosalie Ferner, MD, FRCP, <i>Guy's and St. Thomas' NHS Foundation Trust London</i> ; Cynthia Campen, MD, <i>Stanford University</i> ; Katie Metrock, MD, <i>University of Alabama at Birmingham</i>	
		- Virtual Education Opportunities for NF Clinicians -	
10:10 AM	10:20 AM	<b>European INFER Opportunities</b> Amedeo Azizi, MD, <i>Medical University of Vienna</i>	
10:20 AM	10:30 AM	<b>The US Virtual Case Conference</b> Pamela Trapani, MD, <i>University of Florida at Jacksonville</i>	
10:30 AM	11:30 AM	<b>Case Presentation: Women's Health in NF and Schwannomatosis</b> <b>Moderator:</b> Nicole Ullrich, MD, PhD <b>Panelists:</b> Angela Hirbe, MD, PhD, <i>Washington University</i> ; Jason Picconi, MD, PhD, <i>MercyOne Perinatal Center</i> ; Allison Weisman, MS, CGC, <i>Ann &amp; Robert Lurie Children's Hospital of Chicago</i>	
11:30 AM	11:40 AM	<b>BREAK</b>	Salons A-C
11:40 AM	12:00 PM	<b>OPENING REMARKS &amp; MISSION MOMENT</b> Welcome & Opening Remarks: Annette Bakker, PhD, <i>President, Children's Tumor Foundation</i> Mission Moment: A moderated panel discussion featuring NF patients Moderator: Emily Greaves, <i>Children's Tumor Foundation</i> Patient Panel: NF1: Katie Holt; NF2: Jake Lipe; SWN: Michele Holbrook	Salons D-G

# AGENDA

Saturday · June 24, 2023 (All times are MST)

12:00 PM	12:30 PM	<b>BREAK</b>	Salons A-C
<b>12:30 PM</b>	<b>1:30 PM</b>	<b>KEYNOTE #1: Drugs That Target RAS and Their Relevance to NF1</b> Frank McCormick, PhD, FRSC, <i>University of California, San Francisco</i>	Salons D-G
1:30 PM	2:00 PM	<b>BREAK</b>	Salons A-C
<b>2:00 PM</b>	<b>3:15 PM</b>	<b>SESSION 1: TIPS AND TRICKS FOR SUCCESSFUL PRECLINICAL DRUG ASSAYS</b>  Session Co-Chairs: Pau Castel, PhD, <i>NYU Langone School of Medicine</i> ; Piotr Topilko, PhD, <i>INSERM</i>	Salons D-G
2:00 PM	2:25 PM	<b>Invited Speaker: Screening and <i>In Vivo</i> Validation of Drugs Targeting Cutaneous Neurofibromas</b> Piotr Topilko, PhD, <i>INSERM</i>	
2:25 PM	2:50 PM	<b>Invited Speaker: Tips and Tricks for Successful Drug Trials in Rare Disease</b> Wade Clapp, MD, <i>Indiana University School of Medicine</i>	
2:50 PM	3:15 PM	<b>Invited Speaker:</b> Darren Hargrave, MB, ChB (Hons), MD, <i>University College London</i>	
3:15 PM	3:30 PM	<b>BREAK</b>	Salons A-C
<b>3:30 PM</b>	<b>5:15 PM</b>	<b>SESSION 2: PAIN &amp; ITCH</b>  Session Co-Chairs: Jaishri Blakeley, MD, <i>Johns Hopkins University</i> ; Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital</i>	Salons D-G
3:30 PM	3:55 PM	<b>Invited Speaker: <i>SMARCB1</i> in Schwann Cells Directly Represses the Transcription of Factors That Induce Pain Sensitivity in Sensory Neurons</b> Larry Sherman, PhD, <i>Oregon Health &amp; Science University</i>	
3:55 PM	4:20 PM	<b>Invited Speaker: Nociceptors: Itch and Pain</b> Matthias Ringkamp, MD, PhD, <i>Johns Hopkins University</i>	
4:20 PM	4:45 PM	<b>Invited Speaker: Visualizing Pain by Imaging</b> Thomas Wilson, MD, MPH, <i>Stanford University</i>	
4:45 PM	5:15 PM	<b>Case Study Discussion</b> Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital</i>	
5:15 PM	6:15 PM	<b>Networking – Non-CME Activity</b> Opportunity for networking for Researchers and Clinicians with a new networking opportunity for Advanced Practice Providers	Salons A-C
6:15 PM	6:45 PM	<b>Special Poster Session &amp; Cocktail Reception – Non-CME Activity</b> This special poster session and cocktail reception will be open to both NF Summit and NF Conference attendees: all are welcome. A selection of pre-selected, “patient-friendly” posters will be highlighted.	Princess East & North Foyer
<b>7:00 PM</b>	<b>9:00 PM</b>	<b>Combined NF Conference – NF Summit Welcome &amp; Awards Dinner – Non-CME Activity</b> Evening program to include: • Presentation of the 2023 Friedrich von Recklinghausen Award • Presentation of the 2023 Volunteer of the Year Award	Palomino 1-5

# AGENDA

Sunday · June 25, 2023 (All times are MST)

7:00 AM	8:00 AM	<b>VCEP Symposium: Advances in NF1 Gene Variant Curation – Non-CME Activity</b> Chair: Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital</i>	Salons D-G
7:00 AM	9:00 AM	<b>Sunday Breakfast</b>	Salons A-C
7:00 AM	3:00 PM	<b>CHECK IN &amp; REGISTRATION</b>	Princess South Foyer
8:30 AM	9:30 AM	<b>KEYNOTE #2: A Fly Approach to NF</b> Ross Cagan, PhD, <i>University of Glasgow</i>	Salons D-G
9:30 AM	10:35 AM	<b>SESSION 3: RASOPATHIES, PART I</b>  Session Co-Chairs: Marco Tartaglia, PhD, <i>Bambino Gesù Children's Hospital</i> ; Hilde Brems, PhD, <i>KU Leuven</i>	Salons D-G
9:30 AM	9:55 AM	<b>Invited Speaker: The Selfish Origin of Germline Mutations in the RTK/RAS/MAPK Pathway</b> Anne Goriely, PhD, <i>University of Oxford</i>	
9:55 AM	10:20 AM	<b>Invited Speaker: Molecular Genetics of RASopathies</b> Marco Tartaglia, PhD, <i>Bambino Gesù Children's Hospital</i>	
10:20 AM	10:35 AM	<b>Platform: Targeting K-RAS to Treat Schwann Cell Tumors With NF1 Loss</b> Liang Hu, <i>Cincinnati Children's Hospital Medical Center</i>	
10:35 AM	11:00 AM	<b>BREAK</b>	Salons A-C
11:00 AM	12:00 PM	<b>SESSION 3: RASOPATHIES, PART II</b>  Session Co-Chairs: Marco Tartaglia, PhD, <i>Bambino Gesù Children's Hospital</i> ; Hilde Brems, PhD, <i>KU Leuven</i>	Salons D-G
11:00 AM	11:25 AM	<b>Invited Speaker: Molecular Mechanisms of LZTR1 in RASopathies and Schwannomatosis</b> Pau Castel, PhD, <i>NYU Langone School of Medicine</i>	
11:25 AM	11:40 AM	<b>Platform: Interneurons that BITE, a Cellular Therapy for NF1 High Grade Gliomas</b> Thomas DeRaedt, <i>Children's Hospital of Philadelphia</i>	
11:40 AM	11:55 AM	<b>Platform: Targeting the NF-kappaB Pathway to Treat NF1-Deficient Tumors</b> Stephanie Bouley, PhD, <i>Massachusetts General Hospital</i>	
12:00 PM	1:00 PM	<b>Sunday Lunch</b>	Salons A-C
1:00 PM	2:00 PM	<b>KEYNOTE #3: Gene Engineering for Rare Disease</b> Ivana Trapani, MD, PhD, <i>Telethon Instit of Gen and Medicine, Naples, IT</i>	Salons D-G
2:00 PM	3:30 PM	<b>SESSION 4A: PRECLINICAL DEVELOPMENT: HOW TO CREATE A PATH FOR GENE THERAPY IN NF, PART I (CONCURRENT SESSION)</b>  Session Co-Chairs: Renyuan Bai, MBBS, PhD, <i>Johns Hopkins University</i> ; Deeann Wallis, PhD, PhD, <i>University of Alabama Birmingham</i>	Salons D-G
2:00 PM	2:25 PM	<b>Invited Speaker: Developing an AAV Gene Replacement Therapy for NF1 Tumors</b> Renyuan Bai, MBBS, PhD, <i>Johns Hopkins University</i>	
2:25 PM	2:50 PM	<b>Invited Speaker</b> Jeremie Vitte, PhD, <i>University of California, Los Angeles</i>	
2:50 PM	3:15 PM	<b>Invited Speaker: Considerations for In-Vitro NF Modeling for Gene Therapy</b> Bob Kesterson, PhD, <i>University of Alabama at Birmingham</i>	
3:15 PM	3:30 PM	<b>Platform: Preclinical Development and In Vivo Delivery of Antisense Oligonucleotides for Targeted NF1 Exon 17 Skipping</b> Marc Moore, PhD, <i>National Horizon Centre</i>	

# AGENDA

Sunday · June 25, 2023 (All times are MST)

<b>2:00 PM</b>	<b>3:30 PM</b>	<b>SESSION 4B: NF1 CLINICAL, PART I (CONCURRENT SESSION)</b>	Salons H-I
		Session Co-Chairs: Carlos Romo, MD, <i>Johns Hopkins University</i> ; Rianne Oostenbrink, MD, <i>Erasmus University Rotterdam</i>	
2:02 PM	2:27 PM	<b>Invited Speaker: Harnessing Innate Immune Responses in MPNST Therapeutics</b> Ping Chi, MD, PhD, <i>Memorial Sloan Kettering</i>	
2:27 PM	2:42 PM	<b>Platform: Evidence for Reduced Incidence of Malignant Peripheral Nerve Sheath Tumors in NF1 Adults and Adolescents Under Close Surveillance</b> Eric Legius, MD, PhD, <i>University of Leuven</i>	
2:42 PM	2:57 PM	<b>Platform: Early Detection of Malignant, Pre-malignant and Benign Peripheral Nerve Sheath Tumors with Liquid Biopsy Cell-Free DNA Fragmentomics</b> Taylor Sundby, MD, <i>National Cancer Institute of Health</i>	
2:57 PM	3:12 PM	<b>Platform: The Phase 2 Trial of Selumetinib and Sirolimus for Patients with Unresectable or Metastatic Malignant Peripheral Nerve Sheath Tumors</b> AeRang Kim, MD, PhD, <i>Children's National Hospital</i>	
3:12 PM	3:27 PM	<b>Platform: Association of Serial Serum Cytokine and Chemokine Levels with Plexiform Neurofibroma Characteristics, Symptoms, and Response to Treatment with the MEK Inhibitor Selumetinib</b> Steven Rhodes, <i>Indiana University</i>	
3:30 PM	3:45 PM	<b>BREAK</b>	Salons A-C
<b>3:45 PM</b>	<b>4:45 PM</b>	<b>SESSION 4B: NF1 CLINICAL, PART II (CONCURRENT SESSION)</b>	Salons H-I
		Session Co-Chairs: Carlos Romo, MD, <i>Johns Hopkins University</i> ; Rianne Oostenbrink, MD, <i>Erasmus University Rotterdam</i>	
3:45 PM	4:10 PM	<b>Invited Speaker: ERN GENTURIS Tumor Surveillance Guidelines for Individuals with Neurofibromatosis Type 1</b> Rianne Oostenbrink, MD, <i>Erasmus University Rotterdam</i>	
4:10 PM	4:25 PM	<b>Platform: Detection of Distinct Nodular Lesions on Whole-Body MRI in a High-Risk Population with Neurofibromatosis Type 1</b> Carlos Romo, MD, <i>Johns Hopkins University</i>	
4:25 PM	4:40 PM	<b>Platform: Social Determinants of Health and Neurocognitive and Psychological Outcomes in Youth with NF1</b> Johanna Nielsen, <i>Children's National Hospital</i>	
<b>3:45 PM</b>	<b>5:00 PM</b>	<b>SESSION 4A: CLINICAL DEVELOPMENT: HOW TO CREATE A PATH FOR GENE THERAPY IN NF, PART II (CONCURRENT SESSION)</b>	Salons D-G
		Session Co-Chairs: Renyuan Bai, MBBS, PhD, <i>Johns Hopkins University</i> ; Deeann Wallis, PhD, <i>University of Alabama Birmingham</i>	
3:45 PM	4:10 PM	<b>Invited Speaker: HSV1 Gene Therapy - Topical Gene Therapy in EB and Relevance for NF1</b> M. Peter Marinkovich, MD, <i>Stanford University</i>	
4:10 PM	4:35 PM	<b>Invited Speaker: Exon Skipping in DMD and Clinical Translation</b> Linda Popplewell, PhD, <i>Teesside University</i>	
4:35 PM	4:55 PM	<b>Expert Panel Discussion</b> <b>Moderators:</b> Renyuan Bai, MBBS, PhD, <i>Johns Hopkins University</i> ; Deeann Wallis, PhD, <i>University of Alabama Birmingham</i> <b>Panelists:</b> Bruce Korf, MD, <i>University of Alabama at Birmingham</i> ; M. Peter Marinkovich, MD, <i>Stanford University</i> ; Linda Popplewell, PhD, <i>Teesside University</i> ; Bob Kesterson, PhD, <i>Pennington Biomedical Research Center</i> ; Marco Giovannini, MD, PhD, <i>University of California, Los Angeles</i> ; Jeremie Vitte, PhD, <i>University of California, Los Angeles</i>	
<b>5:00 PM</b>	<b>5:15 PM</b>	<b>POSTER OVERVIEW</b>	Salons D-G

# AGENDA

## Sunday · June 25, 2023 (All times are MST)

5:30 PM      7:30 PM      **POSTER SESSION AND COCKTAIL RECEPTION – Non-CME Activity**      Princess East & North Foyer

## Monday · June 26, 2023 (All times are MST)

7:00 AM      1:00 PM      **CHECK IN & REGISTRATION**      Princess South Foyer

7:00 AM      8:30 AM      **INDEPENDENT SATELLITE SYMPOSIUM: Alexion, AstraZeneca Rare-Disease Sponsored Symposium – Non-CME Activity**  
 Independent Optional Satellite  
 Present and discuss real-world experiences of Selumetinib treatments in pediatric patients with NF1, through patient case studies.  
 Learn about long-term treatment with Selumetinib, clinical response measurements, and the role of MDT in the management of patients with NF1 and the decision-making process.  
**Speakers:**  
 Brigitte Widemann, MD, *National Cancer Institute*; Andrea Gross, MD, *National Cancer Institute*; Rianne Oostenbrink, MD, *Erasmus MC, Rotterdam, Netherlands*; Laura Klesse, MD, *UT Southwestern Medical Center*; Allan Belzberg, MD, *The Johns Hopkins Hospital*

Salons H-I

7:00 AM      9:00 AM      **Monday Breakfast**      Salons A-C

8:30 AM      9:30 AM      **KEYNOTE #4: New Models for Drug Screening and New Models**  
 Min Jae (MJ) Song, PhD, *Senior Scientist, National Center for Advancing Translational Sciences*

Salons D-G

9:30 AM      10:30 AM      **SESSION 5A, PART I: NOVEL MODELS (CONCURRENT SESSION)**      Salons D-G  
 Session Co-Chairs: Alison Lloyd, *University College London*;  
 Helen Morrison, PhD, *Leibniz Institute on Aging - Fritz Lipmann Institute*

9:30 AM      9:55 AM      **Invited Speaker: The Role of the Microenvironment in Neurofibroma Formation – New Targets for Prevention and Treatment**  
 Alison Lloyd, *University College London*

9:55 AM      10:15 AM      **Invited Speaker: The Development and Validation of a Patient-Derived 3D Meningioma Cell Culture Model**  
 Laurien Van De Weijer, MSc, *University of Plymouth Peninsula Medical School*

10:15 AM      10:30 AM      **Platform: Merlin-deficient iPSCs Show Altered Pluripotency and Constitute a Potential In Vitro Model for NF2-Related Schwannomas**  
 Núria Catasús, PhD, *Hospital Universitari Germans Trias i Pujol*

9:30 AM      10:30 AM      **SESSION 5B: NF2/SWN CLINICAL, PART I (CONCURRENT SESSION)**      Salons H-I  
 Session Co-Chairs: Jaishri Blakeley, MD, *Johns Hopkins University*;  
 P. Leia Nghiemphu, MD, *University of California, Los Angeles*

9:35 AM      9:55 AM      **Invited Speaker: Efficient Pathways to Confirm Schwannomatosis Diagnoses and Making Sense of Gene Variants**  
 Alicia Gomes, MS, CGC, *University of Alabama at Birmingham*

9:55 AM      10:15 AM      **Platform: Genetic Findings in People with at Least One Non-Vestibular Schwannoma and Not Meeting Clinical Criteria for NF2-Related Schwannomatosis**  
 Miriam Smith, PhD, *University of Manchester*

10:15 AM      10:30 AM      **Invited Speaker: Strategies for Clinical Trials for LZTR1, SMARCB1-Related and Other Schwannomatoses**  
 Jaishri Blakeley, MD, *Johns Hopkins University*

10:30 AM      11:00 AM      **BREAK**      Salons A-C

# AGENDA

Monday · June 26, 2023 (All times are MST)

<b>11:00 AM</b>	<b>12:00 PM</b>	<b>SESSION 5A, PART II: NOVEL TARGETS (CONCURRENT SESSION)</b>	Salons D-G
		Session Co-Chairs: Alison Lloyd, <i>University College London</i> ; Helen Morrison, PhD, <i>Leibniz Institute on Aging - Fritz Lipmann Institute</i>	
11:00 AM	11:25 AM	<b>Invited Speaker: Protein Replacement Therapy for Treatment of Schwann Cell Nerve Sheath Tumours</b> Helen Morrison, PhD, <i>Leibniz Institute on Aging - Fritz Lipmann Institute (FLI)</i>	
11:25 AM	11:45 AM	<b>Invited Speaker: Nitrated Proteins: A Novel Category of Tumor-Directed Therapeutic Targets</b> Maria Franco, PhD, <i>Florida International University</i>	
11:45 AM	12:00 PM	<b>Platform: Targeted STAT1 Therapy for the Treatment of LZTR1-Driven Peripheral Nerve Tumors</b> Tonci Ivanisevic, <i>VIB-KU Leuven Center for Cancer Biology</i>	
<b>11:00 AM</b>	<b>12:00 PM</b>	<b>SESSION 5B: NF2/SWN CLINICAL, PART II (CONCURRENT SESSION)</b>	Salons H-I
		Session Co-Chairs: Jaishri Blakeley, MD, <i>Johns Hopkins University</i> ; P. Leia Nghiemphu, MD, <i>University of California, Los Angeles</i>	
11:00 AM	11:15 AM	<b>Platform: Phase II Study of Axitinib in Patients with NF2 and Progressive Vestibular Schwannomas</b> Mekka Garcia, MD, <i>NYU Langone</i>	
11:15 AM	11:35 AM	<b>Invited Speaker: Neuromodulation for the Treatment of Chronic Tinnitus</b> Amber Leaver, <i>Northwestern University</i>	
11:35 AM	11:55 AM	<b>Platform: INTUITT-NF2, An Adaptive Platform-Basket Trial for NF2-Related Schwannomatosis Patients With Progressive Tumors: Primary Outcome of the Brigatinib Treatment Arm</b>	
<b>12:00 PM</b>	<b>12:45 PM</b>	<b>Monday Lunch</b>	Salons A-C
<b>12:45 PM</b>	<b>1:45 PM</b>	<b>KEYNOTE #5: Neuron-Glial Interactions in NF1: Implications for Cognition and Tumorigenesis</b> Michelle Monje, MD, PhD, <i>Stanford University</i>	Salons D-G
<b>1:45 PM</b>	<b>2:45 PM</b>	<b>SESSION 6: ALIGNING PRECLINICAL STUDIES WITH CLINICAL TRIAL OUTCOME MEASURES, PART I</b>	Salons D-G
		Session Co-Chairs: Cristina Fernandez-Valle, PhD, <i>University of Central Florida</i> ; Matthias Karajannis, MD, MS, <i>Memorial Sloan Kettering</i>	
1:45 PM	2:05 PM	<b>Invited Speaker: NF2-Related Schwannomatosis Clinical Trials: Lessons Learned and Future Perspective</b> Matthias Karajannis, MD, MS, <i>Memorial Sloan Kettering</i>	
2:05 PM	2:25 PM	<b>Invited Speaker: Designing a Drug Screening Strategy: Review of Synodos: Lessons Learned &amp; Improvements: CUDC907/Fimepinostat</b> Cristina Fernandez-Valle, PhD, <i>University of Central Florida</i>	
2:25 PM	2:45 PM	<b>Panel Discussion</b> <b>Panelists:</b> Jaishri Blakeley, MD, <i>Johns Hopkins University</i> ; Matthias Karajannis, MD, MS, <i>Memorial Sloan Kettering</i> ; Oliver Hanemann, MD, <i>University of Plymouth</i>	
2:45 PM	3:15 PM	<b>BREAK</b>	Salons A-C
<b>3:15 PM</b>	<b>4:45 PM</b>	<b>SESSION 6: ALIGNING PRECLINICAL STUDIES WITH CLINICAL TRIAL OUTCOME MEASURES, PART II</b>	Salons D-G
		Session Co-Chairs: Cristina Fernandez-Valle, PhD, <i>University of Central Florida</i> ; Matthias Karajannis, MD, MS, <i>Memorial Sloan Kettering</i>	

# AGENDA

Monday · June 26, 2023 (All times are MST)

		<b>SCHWANNOMA CELL AND ANIMAL MODELS: CURRENT AND FUTURE</b>	
3:15 PM	3:25 PM	<b>Invited Speaker: Pros and Cons of Working with Primary Cells Isolated From Meningiomas &amp; Schwannomas</b> Liyam Laraba, PhD, <i>University of Plymouth</i>	
3:25 PM	3:35 PM	<b>Invited Speaker: GEM &amp; GEP Schwannoma Models-Ease and Patient Performance Predictability</b> Marco Giovannini, MD, PhD, <i>University of California, Los Angeles</i>	
3:35 PM	3:45 PM	<b>Speaker: Modeling NF1 Peripheral Nervous System Tumors Using iPSCs</b> Edu Serra, PhD, <i>Germans Trias i Pujol Research Institute</i>	
3:45 PM	4:10 PM	<b>Panel Discussion</b> <b>Panelists:</b> Long-Sheng Chang, PhD, <i>Nationwide Children's Hospital</i> ; Marco Giovannini, MD, PhD, <i>University of California, Los Angeles</i> ; Bob Kesterson, PhD, <i>Pennington Biomedical Research Center</i> ; Liyam Laraba, PhD, <i>University of Plymouth</i> ; Lu Le, MD, PhD, <i>University of Texas Southwestern</i> ; Helen Morrison, PhD, <i>Leibniz Institute on Aging</i>	
		<b>MENINGIOMA CELL AND ANIMAL MODELS; NOVEL IN VITRO ASSAYS: CURRENT AND FUTURE</b>	
4:10 PM	4:20 PM	<b>Speaker: Meningioma Cell, Organoid, and Animal Models for Preclinical Evaluation</b> Long-Sheng Chang, PhD, <i>Nationwide Children's Hospital</i>	
4:20 PM	4:30 PM	<b>Speaker: 3D Meningioma Spheroids to Bridge the Translational Gap Between <i>In Vitro</i> and <i>In Vivo</i> Studies</b> Laurien van de Weijer, MSc, <i>University of Plymouth Peninsula Medical School</i>	
4:30 PM	4:45 PM	<b>Panel Discussion</b> <b>Panelists:</b> Long-Sheng Chang, PhD, <i>Nationwide Children's Hospital</i> ; Oliver Hanemann, MD, <i>University of Plymouth</i> ; Bob Kesterson, PhD, <i>Pennington Biomedical Research Center</i> ; Liyam Laraba, PhD, <i>University of Plymouth</i> ; Lu Le, MD, PhD, <i>University of Texas Southwestern</i> ; Edu Serra, PhD, <i>Germans Trias i Pujol Research Institute</i>	
4:45 PM	5:00 PM	<b>BREAK</b>	Salons A-C
<b>5:00 PM</b>	<b>7:00 PM</b>	<b>SESSION 7B: PLATFORM SESSION – BASIC SCIENCE (CONCURRENT SESSION)</b>	Salons H-I
5:00 PM	5:15 PM	<b>Platform: Contribution of Fibroblasts to Tumor Growth in 3D NF1 Plexiform Neurofibroma Cultures</b> Kyugmin Ji, PhD, <i>Henry Ford Health Center</i>	
5:15 PM	5:30 PM	<b>Platform: Aberrant Accumbal Neurocircuitry Underlies ADHD Phenotype in Translational Model of Neurofibromatosis Type 1</b> Jodi Lukkes, PhD, <i>Indiana University School of Medicine</i>	
5:30 PM	5:45 PM	<b>Platform: Single-Cell Sequencing Reveals Transcriptomic Diversity That Facilitates the Malignant Transformation of NF1 Nerve Sheath Tumors</b> Xiyuan Zhang, PhD, <i>National Institute of Health</i>	
5:45 PM	6:00 PM	<b>Platform: Combining Brigatinib with mTOR Inhibition to Effectively Treat NF2-Deficient Meningiomas and MPNST</b> Janet Oblinger, PhD, <i>Abigail Wexner Research Institute at Nationwide Children's Hospital</i>	
6:00 PM	6:15 PM	<b>Platform: Conditioned Media From Painful Human Schwannomatosis Tumors Hypersensitize Sensory Neurons to Painful Stimuli; An <i>In Vitro</i> and <i>In Vivo</i> Study</b> Kimberley Ostrow, PhD, <i>Johns Hopkins University</i>	
6:15 PM	6:30 PM	<b>Platform: WP1066 Induces Cell Death in a Schwannomatosis Patient-Derived Schwannoma Cell Line</b> Abdulrahman Allaf, <i>University of Miami Miller School of Medicine</i>	
6:30 PM	6:45 PM	<b>Platform: CDK4/6-MEK Inhibition in MPNSTs Causes Plasma Cell Infiltration, Sensitization to PD-L1 Blockade, and Tumor Regression</b> Joshua Lingo, <i>University of Iowa</i>	
6:45 PM	7:00 PM	<b>Platform: Integrated Analysis of Cell-Free DNA for the Detection of Malignant Peripheral Nerve Sheath Tumors in Patients with Neurofibromatosis Type I</b> Derek Wong, PhD, <i>Princess Margaret Cancer Center</i>	

# AGENDA

## Monday · June 26, 2023 (All times are MST)

5:00 PM	7:00 PM	<b>SESSION 7A: PLATFORM SESSION – CLINICAL SCIENCE (CONCURRENT SESSION)</b>	Salons D-G
5:00 PM	5:15 PM	<b>Platform: Improving Measurement of Quality of Life in NF2 Clinical Trials: Analysis of INTUITT-NF2 Participant Interviews and Patient-Reported Outcomes</b> Liesel Von Imhof, <i>Massachusetts General Hospital</i>	
5:15 PM	5:30 PM	<b>Platform: Single-Cell Transcriptomic Analysis of NF2-Associated Schwannomas Reveals Novel Changes in Tumor and Immune Cell Subpopulations After Bevacizumab Treatment</b> Long-Sheng Chang, PhD, <i>Nationwide Children's Hospital</i>	
5:30 PM	5:45 PM	<b>Platform: Identification of Immune-Related Candidate Biomarkers in Plasma of Patients with Sporadic Vestibular Schwannoma</b> Konstantina Stankovic, MD, PhD, <i>Stanford University School of Medicine</i>	
5:45 PM	6:00 PM	<b>Platform: Evaluation of cNF Burden and its Impact on Quality of Life in People With NF1 Using 3D Whole-Body Photographs and Modified-Skindex</b> Mandi Johnson, <i>Johns Hopkins University School of Medicine</i>	
6:00 PM	6:15 PM	<b>Platform: High Intensity Focused Ultrasound (HIFU) treatment of Cutaneous Neurofibromas (cNF): Preliminary Results From a Prospective Dual-Center Clinical Investigation</b> Katrine Karmisholt, MD, PhD, <i>Bispebjerg University Hospital</i>	
6:15 PM	6:30 PM	<b>Platform: TRAM-01: A Phase 2 Study of Trametinib for Pediatric Patients With Neurofibromatosis Type 1 and Plexiform Neurofibromas</b> Sebastien Perreault, MD, <i>CHU Sainte-Justine</i>	
6:30 PM	6:45 PM	<b>Platform: Auditory Dysfunction Among Individuals With Neurofibromatosis Type 1 and Their Treatment in Children</b> Alice Maier, <i>Murdoch Children's Research Institute</i>	
6:45 PM	7:00 PM	<b>Platform: MRI Shape and Intensity Features are Associated with Vision Loss in Children with NF1-OPG</b>	

## Tuesday · June 27, 2023 (All times are MST)

7:00 AM	9:00 AM	<b>Tuesday Breakfast</b>	Salons A-C
8:30 AM	9:30 AM	<b>SESSION 8: POSTER COMPETITION – ORAL PRESENTATIONS</b>	Salons D-G
9:30 AM	11:00 AM	<b>SESSION 9A: LATE BREAKING ABSTRACTS (CONCURRENT SESSION)</b>	Salons D-G
9:30 AM	9:50 AM	<b>Platform: Can MEK Inhibitors Help to Avoid the Need for Surgery in Neurofibromatosis Type 1 (NF1) with a Spinal Phenotype</b> Alexander Lee, MD, PhD, <i>The Christie NHS Foundation Trust</i>	
9:50 AM	10:10 AM	<b>Platform: Magnetic Resonance Elastography Predicts Tumor Composition, Behavior, and Patient Outcomes in Vestibular Schwannomas</b> Bailey Duhon, <i>The Ohio State University, Wexner</i>	
10:10 AM	10:30 AM	<b>Platform: Impact of Fibroblasts on Cell Proliferation of NF2-Mutant Schwann Cells and Tumor Progression in Vestibular Schwannoma</b> Olena Bracho, <i>University of Miami, Miller School of Medicine</i>	
10:30 AM	10:50 AM	<b>Platform: Developmental Trajectories in Infants and Pre-School Children with Neurofibromatosis 1</b> Shruti Garg, MD, PhD, <i>University of Manchester</i>	



# AGENDA

Tuesday · June 27, 2023 (All times are MST)

<b>9:00 AM</b>	<b>11:00 AM</b>	<b>SESSION 9B: INDUSTRY PLATFORMS (CONCURRENT SESSION) – Non-CME Activity</b>	Salons H-I
9:00 AM	9:15 AM	<b>Platform: Treatment Patterns and Healthcare Resource Utilization of Pediatric Patients with Neurofibromatosis Type 1-Associated Symptomatic Inoperable Plexiform Neurofibroma in the United States</b> <i>Theresa Dettling, Alexion, AstraZeneca Rare Disease</i>	
9:15 AM	9:30 AM	<b>Platform: HLX-1502: A Novel Potential Treatment for NF Type 1 Plexiform Neurofibromas</b> <i>Emma Davies, PhD, Healx Ltd</i>	
9:30 AM	9:45 AM	<b>Platform: TEAD Autopalmitoylation Inhibitors Prevent NF2-Deficient Meningioma Growth in an <i>In Vivo</i> Skull Convexity Model</b> <i>Liyam Laraba, PhD, University of Plymouth</i>	
9:45 AM	10:00 AM	<b>Platform: Targeting NF2-Deficient Tumors with VT3989</b> <i>Andrew Dorr, MD, Vivace Therapeutics</i>	
10:00 AM	10:15 AM	<b>Platform: NVD-003, an Osteogenic Cell-Based Bone Graft Derived from Autologous Adipose Tissue, Used in the Treatment of Congenital Pseudarthrosis of the Tibia</b> <i>Phillip McClure, MD, FAAOS, Sinai Hospital of Baltimore</i>	
10:15 AM	10:30 AM	<b>Platform: A Population Pharmacokinetic Assessment of the Effect of Food on Selumetinib in Patients With Neurofibromatosis Type 1-Related Plexiform Neurofibromas and Healthy Volunteers</b> <i>Million Arefayene, Alexion AstraZeneca Rare Disease</i>	
10:30 AM	10:45 AM	<b>Platform: PRG-N-01, a Selective Inhibitor of T<math>\beta</math>R1-RKIP Interaction, Suppresses Schwannoma Formation in NF2 Mouse Model</b> <i>Yeoun-Ho Chung, MD, PRG S&amp;Tech Inc.</i>	
10:45 AM	11:00 AM	<b>Platform: 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</b> <i>Scott Plotkin, MD, PhD, Massachusetts General Hospital</i>	
11:00 AM	11:15 AM	<b>BREAK</b>	Salons A-C
<b>11:15 AM</b>	<b>1:15 PM</b>	<b>CONSORTIA AND COLLABORATION UPDATES</b>	Salons D-G
11:15 AM	11:40 AM	<b>Invited Speaker: NF Clinical Trials Consortium</b> <i>Michael Fisher, MD, Children's Hospital of Philadelphia</i>	
11:40 AM	12:00 PM	<b>Invited Speaker: NF1 Optic Pathway Glioma Natural History Study</b> <i>Robert Avery, DO, MSCE, Children's Hospital of Philadelphia</i>	
12:00 PM	12:25 PM	<b>Invited Speaker: Genomic Blood-Based Biomarker to Improve Cancer Detection in NF1: A Collaboration Between NIH and Washington University</b> <i>Angela C. Hirbe, MD, PhD, Washington University in St. Louis</i>	
12:25 PM	12:50 PM	<b>Invited Speaker: Neurofibromatosis Therapeutic Acceleration Program (NTAP)</b> <i>Jaishri Blakeley, MD, Johns Hopkins University</i>	
12:50 PM	1:15 PM	<b>Invited Speaker: Gilbert Family Foundation</b> <i>YooRi Kim, MS, Scientific Officer, Gilbert Family Foundation</i>	
<b>1:15 PM</b>	<b>1:30 PM</b>	<b>FAREWELL AND ADJOURNMENT</b>	Salons D-G



**Fairmont Gold:**

6101 - 6114 | 6201 - 6218  
6301 - 6316 | 6401 - 6424

**Building B:**

1082 - 1118 | 2062 - 2118  
3082 - 3115 | 4082 - 4098

**Building C:**

1119 - 1144 | 2119 - 2144  
3119 - 3141

**Building D:**

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3038 - 3060 | 4038 - 4060

**Building F:**

1014 - 1037 | 2006 - 2037  
3001 - 3037 | 4001 - 4021

**Building G:**

1201 - 1220 | 2201 - 2220  
3201 - 3220

**Sunset Beach Building:**

1401 - 1434 | 2401 - 2434  
3401 - 3434

**Casitas II:**

5200 - 5209  
5110 - 5121  
5122 - 5131  
5132 - 5141

**Casitas III:**

5300 - 5309  
5310 - 5321  
5322, 24, 26, 28, 30  
5332, 34, 36, 38, 40

**Casitas IV:**

5400 - 5409  
5410, 12, 14, 16, 18

- 36. East Palomino Plaza
- 37. Sunset Lawn
- 38. Princess Pool Upper East Deck
- 39. Princess Pool Upper West Deck
- 40. Sunset Beach Building
- 41. Sunset Beach Pool
- 42. Copper Canyon Western Town

- 28. Sonoran Splash Pool
- 29. Sport Court & Playground
- 30. Sonoran Landing Pool
- 31. Toro Latin Restaurant & Rum Bar at TPC  
(complimentary shuttle at front drive)
- 32. Ironwood Meeting Room\*
- 33. Pavilion (seasonal)
- 34. North Palomino Plaza
- 35. Saguaro Boardroom

- 19. Well & Being Salon
- 20. Casita Pool & Jacuzzi
- 21. Princess Pool & Bar
- 22. Ironwood American Kitchen\*
- 23. Cabana Cafe/Princess Pool Bar/Princess Pool Overlook
- 24. Trailblazers Family Adventure Center\*
- 25. Fairmont Fitness Center\*
- 26. Fragrance Garden
- 27. Ironwood Circle

- 10. Princess Plaza
- 11. The Plaza Bar
- 12. Princess Falls\*
- 13. Princess Ballroom & Conference Center
- 14. Palomino Ballroom & Conference Center
- 15. La Hacienda by Richard Sandoval
- 16. Well & Being Spa
- 17. Hacienda Plaza & Trellis
- 18. Canyon Lawn

- 1. Lobby
- 2. Front Desk
- 3. Bell Desk
- 4. Concierge Desk/Golf Concierge
- 5. Car Rental
- 6. Provisions Retail Market
- 7. Mawerick Menswear
- 8. Anna J Boutique
- 9. Bourbon Steak by Michael Mina

**Casita Meeting Rooms**

- ▲ I 5130
- ▲ II 5232
- ▲ III 5308
- ▲ III 5320
- ▲ III 5332
- ▲ IV 5400

\*Lower Level **E** Elevator

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**Verena Staedtke, MD, PhD**, Johns Hopkins University

**Piotr Topilko, PhD**, L'Institut national de la santé et de la recherche médicale (INSERM)

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