

PROGRAM BOOK

# 2024 GLOBAL NF CONFERENCE

June 20-25, 2024

The EGG Brussels  
Belgium



Organized by:



Hosted by:





Dear NF Conference Attendees:

On behalf of the Children's Tumor Foundation (CTF), the European NF Group, and the CTF Europe, welcome to the 2024 Global NF Conference in glorious Brussels, the historic capital of Belgium. Brussels, the capital and administrative center of the European Union, serves as a crossroads for political and commercial activity in Europe. But, for our community over the next five days, Brussels will serve as the global forum for progress in neurofibromatosis and schwannomatosis.

This event is unique as it is not 'just a conference,' it is a gathering of all stakeholders—academia, industry, government, and most importantly, patients—who bring unique approaches and perspectives to ending NF. It will be a celebration of science, a celebration of our resilient patient community, and an opportunity for mentoring and connection for new researchers in the field.

When the first Global NF Conference was held in Paris in 2018, it became evident that a joint meeting with the Children's Tumor Foundation and the European NF group on European soil would expand NF's global footprint, attracting participants from the far reaches of the globe, granting more people the opportunity to gain knowledge and establish connections, which is often difficult to attain for many. We are eager to meet and make many new friends!

I would like to congratulate our European/American planning committee this year. **Hilde Brems** of Belgium, **Ignacio Blanco Guillermo** of Spain, and **Justin Jordan** and **Laura Klesse** of the USA, organized an exceptional meeting. They were a joy to work with and exemplified fellowship, scholarship, and teamwork. This meeting will, for the first time, allocate a full-day session to each of the main topics, allowing for a deeper dive into the subject matter. Keynotes by top-notch experts outside of NF, will be followed by a perspective from top NF'ologists who will weave in outside-the-box thinking into applications for NF, and giving more time for community discussion.

I am honored to share that some high-level European officials have agreed to address our community:

- First, **Dr. Nathalie Moll**, Director General, EFPIA - European Federation of Pharmaceutical Industries and Associations (EFPIA) has agreed to open our conference.
- On the day of the new therapeutic modalities, **Dr. Niklas Blomberg**, Executive Director of Innovative Health Initiative, and **Magda Chlebus**, Executive Director Scientific & Regulatory Affairs at EFPIA and a Board member of CTF Europe, will participate in a fireside chat discussing the unique public-private partnership and other rare disease opportunities in Europe.

The conference co-chairs selected top keynote speakers with diverse backgrounds:

**Luigi Naldini, MD, PhD**, from San Raffaele University (Italy) will deliver a keynote on a non-NF Gene Therapy effort from "*Bench to Bedside and the Market: A Roadmap to the Development of New Advanced Gene and Cell Therapies.*"

The Comprehensive Care Keynote speaker, **Abby Rosenberg, MD**, from Boston Children's Hospital, Harvard University (USA) will discuss "*Science and the Art of Resilience: 5 Lessons Learned from Patients, Communities, and Society.*"

**Andrew Rice, MD**, from Imperial College London (UK) will provide an "*Update on the Diagnosis And Management of Chronic Neuropathic Pain*" for the SWN session.

For the Novel Therapeutics session, **Dan Nomura, PhD**, from University of California, Berkeley (USA) will present a talk about "*Reimagining Druggability using Chemoproteomic Platforms.*"

Finally, **Casey Greene, PhD**, from University of Colorado (USA) will deliver the final keynote of the meeting on "*Engineering Serendipity: AI's Rapidly Expanding Role in Research and Care*" for the AI, Novel Technologies, Biomarkers session.

To underline the uniqueness of our gathering, I want to thank the respective planning committees for organizing the first-ever Young Investigator Day and Patient Day before the conference. We also want to encourage you to look for the highly interesting satellite meetings on very specific topics that have been organized by our long-term friends and partners.

This year's meeting will open with a heartfelt tribute by our honorary chair Dr. Eric Legius, remembering our dear friend and colleague, Ludwine Messiaen, PhD, to whom we dedicate the 2024 Global NF Conference.

I look forward to seeing you this week and hope the program inspires and motivates you in the year ahead. This conference is one of CTF's highlights. Every year I become further convinced that the community gathered here will help us achieve our mission to end NF!



**Annette Bakker, PhD, President**

Children's Tumor Foundation



Network: Make NF Research Visible  
Password: endnf2024

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### LAY GROUP REPRESENTATIVES

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\*up to date through June 8, 2024

# SCHEDULE

## Schedule At-A-Glance

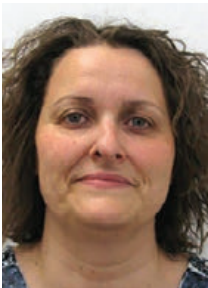
	TIME	EVENT	LOCATION
<b>THURSDAY</b> 20 JUNE	7:30 - 17:00	Registration & Check In	Reception
	9:30 - 13:00	Young Investigator Day – Morning Session – By Invitation Only	Studio
	14:00 - 17:00	REiNS 2024 Summer Meeting	Auditorium
	14:00 - 18:00	Young Investigator Day – Afternoon Session – By Invitation Only	Studio
	18:00 - 21:00	Young Investigator Day – Evening Session – By Invitation Only	Studio
<b>FRIDAY</b> 21 JUNE	7:30 - 17:00	Registration & Check In	Reception
	8:30 - 11:30	<b>CLINICAL CARE SYMPOSIUM</b>	Auditorium
	9:00 - 16:00	Patient Day Program – Open to NF Patients and Lay Groups (Patient Advocates)	Studio
	9:00 - 10:30	Workshop: Requesting, Reusing, and Analyzing Data on the NF Data Portal	Cinema
	10:30 - 12:00	Workshop: Data Curation with Large Language Models	Cinema
	11:30 - 12:15	Lunch Break	Riverside
	12:15 - 13:00	<b>OPENING REMARKS</b>	Auditorium
	13:00 - 15:25	<b>GENE THERAPY</b>	Auditorium
	15:25 - 16:30	<b>KEYNOTE: Gene Therapy</b>	Auditorium
	16:30 - 16:45	Session Perspective: Gene Therapy	Auditorium
	16:45 - 17:45	Presentation of Awards	Auditorium
	18:30 - 21:30	<b>Welcome Reception</b>	Royal Museums of Fine Arts of Belgium
	<b>SATURDAY</b> 22 JUNE	7:00 - 17:00	Registration & Check In
7:15 - 8:45		Navigating Transitions of Care in Patients With NF1-PN: A Case-Based Discussion (Satellite Symposium)	Studio
9:00 - 10:00		<b>KEYNOTE: Comprehensive Care</b>	Auditorium
10:15 - 10:30		Session Perspective: Comprehensive Care	Auditorium
10:30 - 12:00		<b>COMPREHENSIVE CARE – Morning Session</b>	Auditorium
12:00 - 13:00		Lunch Break	Riverside
13:00 - 14:30		<b>COMPREHENSIVE CARE – Afternoon Session</b>	Auditorium
14:30 - 15:30		Consortia Updates	Auditorium
15:30 - 17:30		Combined Poster Sessions & Cocktail Reception	Grand Hall
17:30 - 20:30	cNF Satellite Meeting: Translational and Clinical Advances for Cutaneous Neurofibroma	Studio	
<b>SUNDAY</b> 23 JUNE	7:00 - 17:00	Registration & Check In	Reception
	7:15 - 8:45	Caregiver & Healthcare Professional Perspectives on the Continuity of Care in NF1-PN (Satellite Symposium)	Studio
	9:00 - 10:00	<b>KEYNOTE: SWN</b>	Auditorium
	10:15 - 10:30	Session Perspective: Schwannomatosis	Auditorium
	10:30 - 12:05	<b>SCHWANNOMATOSIS – Morning Session</b>	Auditorium
	12:05 - 13:00	Lunch Break	Riverside
	13:00 - 15:00	<b>SCHWANNOMATOSIS – Afternoon Session</b>	Auditorium
	15:00 - 17:00	<b>BASIC / PRECLINICAL PLATFORM SESSION</b>	Auditorium
<b>MONDAY</b> 24 JUNE	7:30 - 17:00	Registration & Check In	Reception
	8:00 - 9:00	<b>LATE BREAKING ABSTRACTS</b>	Auditorium
	9:00 - 10:00	<b>KEYNOTE: Novel Therapeutics</b>	Auditorium
	10:10 - 10:15	Session Perspective: Novel Therapeutics	Auditorium
	10:15 - 12:00	<b>NOVEL THERAPEUTICS – Morning Session</b>	Auditorium
	12:00 - 12:55	Optional Lunch Satellite: VCEP	Studio
	13:00 - 15:00	<b>NOVEL THERAPEUTICS – Afternoon Session</b>	Auditorium
	15:00 - 17:00	<b>CLINICAL PLATFORM SESSION</b>	Auditorium
	17:30 - 18:30	Fireside Chat	Studio
<b>TUESDAY</b> 25 JUNE	7:30 - 13:00	Registration & Check In	Reception
	8:00 - 9:00	Poster Competition Winners – Oral Presentations	Auditorium
	9:00 - 10:00	<b>KEYNOTE: AI, Novel Technologies, Biomarkers</b>	Auditorium
	10:15 - 10:30	Session Perspective: AI, Novel Technologies, Biomarkers	Auditorium
	10:30 - 12:00	<b>AI, NOVEL TECHNOLOGIES, BIOMARKERS – Morning Session</b>	Auditorium
	12:00 - 13:00	Lunch Break	Riverside
	13:00 - 14:45	<b>AI, NOVEL TECHNOLOGIES, BIOMARKERS – Afternoon Session</b>	Auditorium
	14:45 - 15:00	<b>ADJOURNMENT</b>	Auditorium

## 2024 NF Conference Co-Chairs



**Ignacio Blanco, MD, PhD**, *Hospital Universitari Germans Trias i Pujol, Spain*

Ignacio Blanco, M.D., Ph.D. practices Clinical Genetics at the Germans Trias Hospital. He serves as Clinical Director of the Clinical Laboratory of the Metropolitan Nord Area of Catalunya and Chairman of the Clinical Genetics Department at the Germans Trias Hospital. Dr. Blanco received his MD degree at the University of Barcelona and his PhD degree at the Autonomous University of Barcelona. Dr. Blanco completed his residency in General and Digestive Surgery at Sant Pau Hospital (Barcelona, Spain), and completed a research fellowship in Medical Oncology and Immunotherapy at University of Nebraska Medical School, Omaha, Nebraska, USA. Dr. Blanco is Spanish board-certified in General and Digestive Surgery and accredited in Human Genetics by the Spanish Society of Human Genetics. Dr. Blanco is Associated Professor of the Department of Surgery at the Universitat Autònoma de Barcelona. Dr. Blanco research activities include emotional Impact of Genetic Counseling, Risk Assessment, rare diseases and Neurofibromatosis. He is the Clinical Leader of the Spanish National Reference Center for adult patients with Neurofibromatosis (CSUR Facomatosis). Dr. Blanco belongs to the Barcelona NF research group together with Dr. C. Lázaro, Dr. E. Serra and Dr. E. Castellanos.



**Hilde Brems, PhD**, *KU Leuven, Belgium*

Hilde Brems graduated as a master in Biomedical Sciences in 2005 and obtained a PhD in Biomedical Sciences in 2010, both at KU Leuven. During her PhD she received an IWT scholarship and studied under the supervision of Prof. Legius a new condition resembling neurofibromatosis type 1, now known as Legius syndrome (autosomal dominant condition caused by a heterozygous mutation in SPRED1). She was awarded in 2012 with the three-yearly prize from the Academische Stichting Leuven for best PhD thesis within Biomedical Sciences at KU Leuven. From 2011 until 2017, she was an FWO-funded postdoctoral researcher at the department of Human Genetics at KU Leuven. During her postdoctoral fellowship she joined the lab of Prof. Elgersma at the Department of Neuroscience in Rotterdam (the Netherlands) for 1 year and investigated cognitive and social behavior in Spred1 and Nf1 mouse models. After her return to Leuven, she rejoined the Laboratory for Neurofibromatosis Research. The research focusses on (1) the mechanisms underlying the social behavioral deficits in RAS-MAPK mouse models for autism spectrum disorder, (2) the role of SPRED1 in melanoma, (3) NF1 mosaicism. Since October 2017 she started as a staff scientist at the Centre for Human Genetics at the University Hospitals Leuven and is responsible for the molecular diagnostics of familial cancer syndromes with a main interest for neurofibromatoses, schwannomatosis and mosaic genodermatoses.



**Justin Jordan, MD, MPH**, *Massachusetts General Hospital, US*

Dr. Jordan is an Assistant Professor of Neurology at Harvard Medical School, and serves as Clinical Director for Neuro-Oncology and Director of the Family Center for Neurofibromatosis at Massachusetts General Hospital. He is a national leader in neurofibromatosis clinical care and research. He leads multiple educational forums for patients and families affected by neurologic disease, including NF, and is a perennial volunteer for patient advocacy organizations. Dr. Jordan serves as a member of the Children's Tumor Foundation Clinical Care Advisory Board, the chair of the scientific advisory board of Neurofibromatosis Network, and is a member of the board of directors for Neurofibromatosis Northeast.



**Laura Klesse, MD, PhD**, *University of Texas Southwestern Medical Center, US*

Dr Laura Klesse is a pediatric neuro-oncologist who specializes in the care of patients with neurofibromatosis and central nervous system tumors. Dr Klesse obtained her PhD in the laboratory of Dr Luis Parada studying the signaling cascades involved in tumor formation in NF1, followed by her pediatric oncology training at UT Southwestern. Dr. Klesse is currently the Director of the Comprehensive Neurofibromatosis Program at UT Southwestern and Children's Health in Dallas, Texas. She serves as the site's principal investigator for the Children's Oncology Group, the National NF Clinical Trials Consortium and the Pediatric Early Phase Clinical Trials Network. She serves as Vice-Chair of the Children's Tumor Foundation's Clinical Care Advisory Board and the co-chair of the UT Southwestern Simmons Cancer Center Protocol Review Board.

## 2024 NF Conference Keynote Speakers



**Casey Greene, PhD**, *University of Colorado, US*

Casey is the Chair and a Professor in the Department of Biomedical Informatics at the University of Colorado School of Medicine. Before moving to Colorado in 2020, he served as the founding Director of the Childhood Cancer Data Lab for Alex's Lemonade Stand Foundation. Since 2021, he has also served as the Interim Director of the Colorado Center for Personalized Medicine, which is responsible for developing scalable strategies to bring genetics to the point of care. His lab develops machine learning methods that integrate distinct large-scale datasets to extract the rich and intrinsic information embedded in such integrated data. This approach reveals underlying principles of an organism's genetics, environment, and response to that environment. The overarching theme of his work has been the development and evaluation of methods that acknowledge the emergent complexity of biological systems.



**Luigi Naldini, MD, PhD**, *San Raffaele University, Italy*

Luigi Naldini, M.D., Ph.D., is Professor of Cell and Tissue Biology and of Gene and Cell Therapy at the San Raffaele University School of Medicine and Scientific Director of the San Raffaele Telethon Institute for Gene Therapy (Milan, Italy). He has received his medical degree from the University of Turin (Italy) and his PhD from the University "La Sapienza" of Rome (Italy). For the past 30 years he has pioneered the development and applications of lentiviral vectors for gene therapy, which have become one of the most widely used tools in biomedical research and are providing a long-sought hope of cures for several otherwise deadly human diseases. Throughout this time, he has continued to investigate strategies to overcome the major hurdles to safe and effective gene therapy, bringing about innovative solutions that are not only being translated into new therapeutic strategies for genetic diseases and cancer, but have

also allowed novel insights into hematopoietic stem cell function and tumor angiogenesis. He also contributed to pioneer and advance the use of artificial nucleases for targeted genome editing in cell and gene therapy. He has published >300 scientific papers. SCOPUS Author h-index: 108.

Member of the European Molecular Biology Organization (EMBO), has been President of the European Society of Gene and Cell Therapy (ESGCT), appointed as expert on the "Human Gene Editing Study" of the US National Academies of Sciences and of Medicine, and on the Italian National Committee for Biosafety, Biotechnology and Life Sciences. He was awarded the Outstanding Achievement Award from the American Society of Gene and Cell Therapy (ASGCT) in 2014 and from ESGCT in 2015, an Honorary doctorate from the Vrije University, Brussel, in 2015, the Jimenez Diaz Prize in 2016, the Beutler Prize from the American Society of Hematology (ASH) in 2017 and the 2019 Jeantet-Collen Prize for Translational Medicine. He was nominated "Grande Ufficiale dell'Ordine Al Merito della Repubblica Italiana", one of the highest-ranking honor in Italy, and elected member of "Accademia dei Lincei", the oldest and most prestigious national academic society. He is also co-founder of three innovative biotech start-up companies: Genenta (recently listed on Nasdaq), Epsilon Bio (now acquired by Chroma Medicine) and Genespire.



**Daniel K. Nomura, PhD**, *University of California, Berkeley, US*

Dan Nomura is a Professor of Chemical Biology and Molecular Therapeutics in the Department of Chemistry and the Department of Molecular and Cell Biology in the Division of Molecular Therapeutics at the University of California, Berkeley and an Investigator at the Innovative Genomics Institute. He is an Adjunct Professor in the Department of Pharmaceutical Chemistry at UCSF. Since 2017, he has been the Director of the Novartis-Berkeley Translational Chemical Biology Institute focused on using chemoproteomic platforms to tackle the undruggable proteome. He is Co-Founder of Frontier Medicines, a start-up company focused on using chemoproteomics and machine learning approaches to tackle the undruggable proteome. He is also the Founder of Vicinitas Therapeutics based on his group's discovery of the Deubiquitinase Targeting Chimera (DUBTAC) platform for targeted protein stabilization. He is on the

Scientific Advisory Boards for Frontier Medicines, Vicinitas Therapeutics, Photys Therapeutics, Apertor Pharma, Ecto Therapeutics, and Oerth Bio. Nomura is also on the scientific advisory boards of The Mark Foundation for Cancer Research and the MD Anderson Cancer Center. He is also an Investment Advisory Partner at a16z Bio+Health, an Investment Advisory Board member at Droia Ventures, and an iPartner with The Column Group. He earned his B.A. in Molecular and Cell Biology in 2003 and Ph.D. in Molecular Toxicology in 2008 at UC Berkeley with Professor John Casida and was a postdoctoral fellow at Scripps Research with Professor Benjamin F. Cravatt before returning to Berkeley as a faculty member in 2011. Among his honors include the National Cancer Institute Outstanding Investigator Award, Searle Scholar, and the Mark Foundation for Cancer Research ASPIRE award.

## 2024 NF Conference Keynote Speakers



**Andrew S.C. Rice, MB BS, MD, FRCP, FRCA**, *Imperial College London, UK*

Andrew S.C. Rice MB BS, MD, FRCP, FRCA is Professor of Pain Research at Imperial College London and Honorary Consultant in Pain Medicine at Chelsea and Westminster Hospital, London, United Kingdom. He received his medical degree from St. Mary's Hospital Medical School in 1982 and his research doctorate from St. Thomas' Hospital Medical School in 1991. He underwent specialist training in Oxford and at St Thomas' Hospital and joined the academic staff of Imperial College in 1995.

His translational research programme seeks to elucidate neuropathic pain and enjoys the benefit of a highly interdisciplinary group of researchers and collaborators. Andrew's research focuses covers both laboratory and clinical research spanning animal models, through deep profiling of patients with a view to personalized medicine, to clinical trials and evidence synthesis by meta-analysis. His focus is on neuropathic pain in the context of infectious diseases (HIV, Herpes Zoster, HTLV-1 and leprosy), diabetic neuropathy and peripheral nerve trauma, especially in a military context. He has been a pioneer in the use of deep clinical phenotyping studies to elucidate stratification approaches to empower the concept of precision medicine for the clinical management of neuropathic pain. He is a vocal advocate for tackling the record of translation failure in his field by improving the internal and external validity of pre-clinical research and for innovating evidence synthesis methods in pre-clinical research. He collaborates with historians to "learn the lessons of history" particularly with regards to post amputation pain in First World War veterans and nutritional neuropathies in survivors of captivity in the Far East 1942-45. He has published ~250 peer reviewed papers and has a citation (H) index of 67.

Andrew chaired the International Association for the Study of Pain Presidential Task Force of Cannabis and Cannabinoid Analgesia and the Scientific Programme Committee of the 2020 World Congress on Pain. He has the privilege of serving as liaison between IASP and our Southeast and South Asian chapters. Having previously served as an IASP Councilor, he was elected President-Elect of IASP in 2022.



**Abby Rosenberg, MD**, *Boston Children's Hospital / Harvard University, US*

Dr. Rosenberg is the Chief of Pediatric Palliative Care at the Dana-Farber Cancer Institute, Director of Palliative Care at Boston Children's Hospital, and Associate Professor of Pediatrics at Harvard Medical School. She received her MD from Stanford University and completed her pediatrics residency and hematology/oncology fellowship training at the University of Washington. Her additional training includes a Master of Science in Clinical Research Methods and a Master of Arts in Bioethics, both from the University of Washington.

Dr. Rosenberg's (U.S.) national leadership positions have included the Chair of the American Society of Clinical Oncology Ethics committee, the Chair of the American Academy of Hospice and Palliative Medicine Scientific Program committee, the Chair of the American Cancer Society's committee for palliative care and outcomes science, Chair of the National Institutes of Health study section for Lifestyle and Health Behaviors, and the Chair of the Children's Oncology Group Palliative Care and Patient-Reported Outcomes committees.

Dr. Rosenberg's scholarship is focused on promoting the quality of life of children, adolescents, and young adults with serious illness. Specifically, she and her team endeavor to create programs to promote patient and family resilience, in turn enabling both children and families to thrive. Dr. Rosenberg has been continuously funded by the U.S. National Institutes of Health (NIH) for her entire career. She has published over 225 peer-reviewed manuscripts related to pediatric palliative/supportive care and survivorship and has been an invited speaker at over 100 national and international meetings. She has received multiple awards for her work, including the 2018 Investigator Award from the American Academy of Hospice and Palliative Medicine, the 2021 Excellence in Mentoring award from the University of Washington, the 2022 Trish Greene Lifetime Achievement Award for Quality of Life Research from the American Cancer Society, and the 2023 Pathfinder in Palliative Care Award, also the American Cancer Society.

Dr. Rosenberg would like to thank the Children's Tumor Foundation, the European NF Group, and CTF Europe for the invitation to join the Global NF community and speak at this incredible venue.



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 (DDI) program is to stimulate NF drug discovery by funding researchers proposing to investigate novel or repurposing therapies for NF or to validate new NF targets. Proposals are expected to be short and concentrated on obtaining key preliminary data needed to quickly advance to the next step of drug discovery.

Specifically, applications must fall into one the following two categories:

- Support early-stage testing of therapeutic compounds for the treatment of NF. Shelved compounds (compounds having successfully undergone clinical testing but not being actively developed further despite this) will be prioritized. For compounds against a specific target (where multiple compounds exist), a strong rationale, also based on safety data, must be provided upon submission.
- Support the validation of a novel NF target. Targets may have been described as potentially relevant in NF or may be de novo. To that end, we encourage the mining of existing publicly available datasets. Target validation experiments should not be limited to the testing of compounds of limited or unknown specificity and should include target engagement experiments using direct biochemical methods or genetic interactions.

### 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).

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:  
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Supported by an educational grant from AstraZeneca



**INFER**  
INTERNATIONAL  
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RESOURCES





## NF RESEARCHERS: WORK WITH OUR PATIENTS

CTF ENGAGE IS THE PATIENT ENGAGEMENT INITIATIVE OF THE CHILDREN'S TUMOR FOUNDATION, dedicated to transforming the landscape of NF research through the curation of quality, meaningful interactions between patients and families with research stakeholders. Our mission is to support scientific projects of merit that generate value for the NF community.

By collaborating with Patient Representatives, you can improve your studies, accelerate the research process, and make vital connections with the NF community.

Patient Representatives are trained in research concepts and regulatory affairs and are prepared to lend their perspectives to your projects. They can:

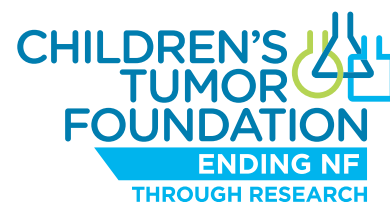
- **Guide research priorities** and help you select a research question that matters to the community
- **Review specific study materials**, such as protocols and recruitment materials
- **Serve as study advisors** (i.e. community advisory boards) and co-investigators
- **Disseminate your results to the community**, such as writing lay summaries and relaying findings at patient events

“The role played by patient representatives has drawn our attention to some of the most important problems faced by NF patients. We are strongly convinced that the continuous interaction between scientists, physicians, and patient advocates will help us immensely to focus on the key unsolved issues on which we should direct our research efforts, in order to build tangible benefits for patients affected by neurofibromatosis.”

ANTONIO IAVARONE, MD  
Professor of Pathology and Cell Biology and Neurology  
Columbia University

If you are looking to work in collaboration with Children's Tumor Foundation Patient Representatives, please reach out to [engage@ctf.org](mailto:engage@ctf.org) to discuss how we can work together.

# CTF Preclinical Hub: Case Studies



The Children's Tumor Foundation (CTF) is accelerating the path to drug discovery by constructing an NF-focused Preclinical Hub to supercharge the development of NF treatments. The Preclinical Hub is built on the successes of the Preclinical Consortium (2008-2016) and Synodos for NF2 initiatives (2014-2017), both of which efficiently delivered treatments to patients. Due to limitations in scalability of both initiatives, CTF decided to expand the Preclinical Hub to become a full public-private partnership.

The Preclinical Hub will speed the approval of potential treatments by offering the following to academic, research, and pharmaceutical industry partners:

- Access to disease models, data tools, drug libraries and biological material
- Expert advice and support during preclinical study design and execution
- Prenegotiated Master Service Agreements
- Predetermined protocols and tests

The Preclinical Hub is already underway and has announced a CTF-funding opportunity to support the generation of clinically relevant models for NF. Additionally, several case studies serve as examples of the function and power of this bold new initiative.

## Case Studies

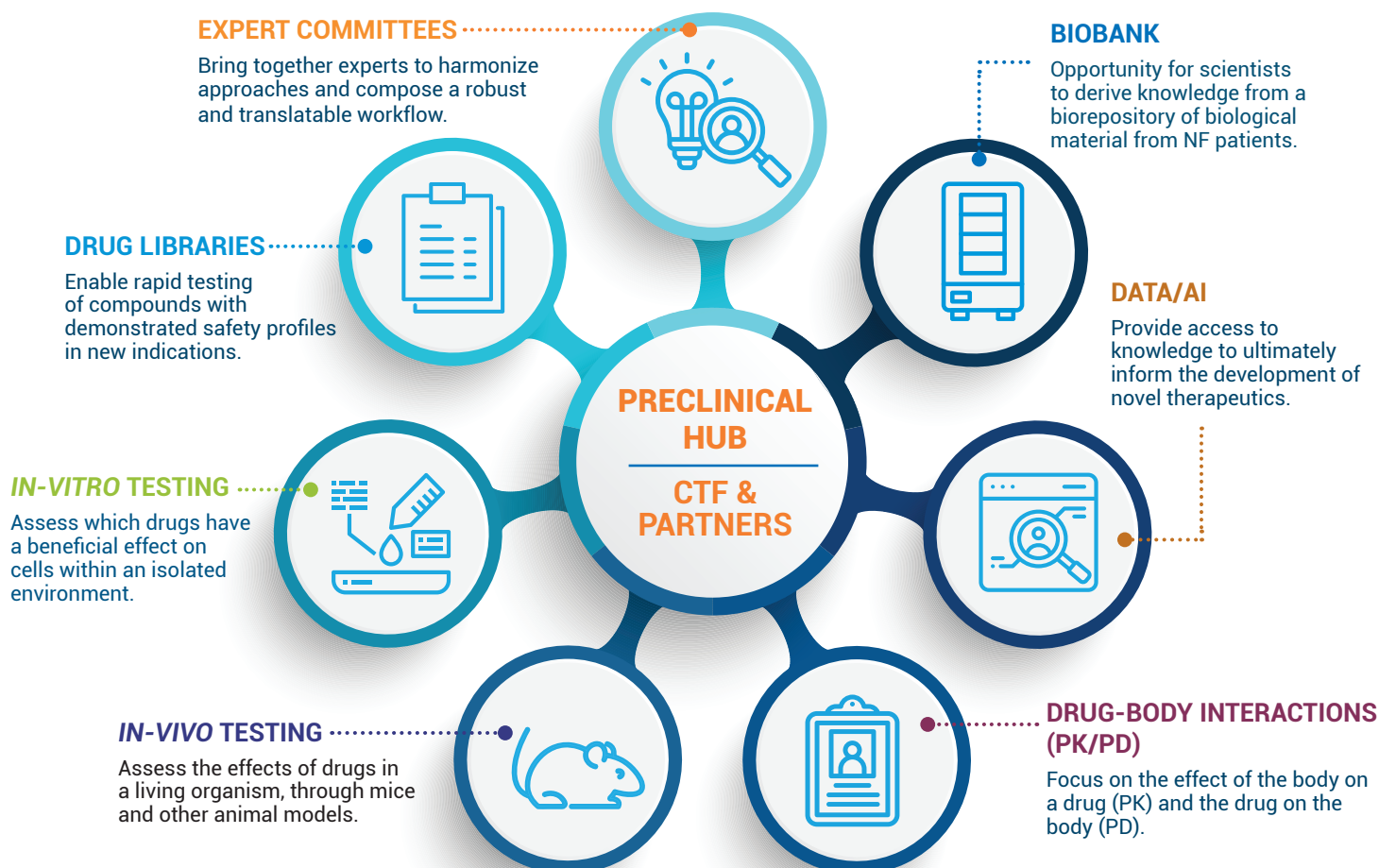
As part of the Preclinical Hub efforts, CTF was able to connect an AI-powered drug discovery company in the rare disease space with researchers holding key preclinical models for NF. Making this connection and sharing our prenegotiated workflows between the company and the expert research facility running the preclinical experiments is a great way to streamline drug development efforts. This will significantly shorten the timeline to the clinic.

We have also helped companies initiate an NF program and support early proof of concept studies that could ultimately lead

to a clinical program for NF. In such cases, working through the Preclinical Hub has provided expert advice, connections to appropriate research experts running preclinical models, and resources for gathering the initial data necessary to potentially launch an NF program.

These examples are an early glimpse into what is possible with the Preclinical Hub initiative. We are delighted to share this progress as we continue to identify, validate, and share the most robust preclinical models for clinical translation, and accelerate the identification of clinical trial-ready therapeutics for NF.

To find out more, visit [ctf.org/preclinicalhub](http://ctf.org/preclinicalhub)



# WOMEN IN NF



## ESSAYISTS INCLUDE:

Margaret “Peggy” Wallace, PhD  
Meena Upadhyaya, OBE,  
FRCPATH, FLSW  
Corina Anastasaki, PhD  
Dusica Babovic-Vuksanovic, MD  
Annette Bakker, PhD  
Patricia Birch, BSc, MSc, RN  
Rosalie Ferner, MD, FRCP  
Sue Huson, MD, FRCP  
Karin Soares Cunha, PhD  
Conxi Lazaro, PhD  
Kathryn North, AC, BSc, MBBS,  
MD, DMedSc  
Minna Pöyhönen, MD, PhD  
Sirkku Peltonen, MD, PhD  
Miriam J Smith, PhD  
Anat Stemmer-Rachamimov, MD  
Nancy Ratner, PhD  
Elizabeth “Betty” Schorry, MD  
Kristine S. Vogel, PhD  
Deeann Wallis, PhD  
Adrienne L. Watson, PhD  
Brigitte Widemann, MD  
Katharina Wimmer, PhD

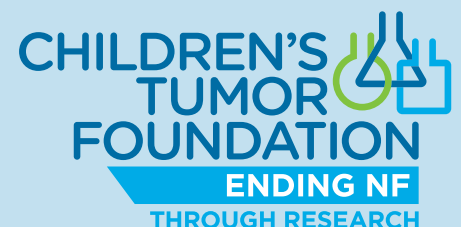
Join us on a journey into the heart and history of NF basic and clinical research with “Women in NF,” a new series of essays submitted by women who have been involved in shaping the landscape of NF understanding and treatment. Launching during the 2024 Global NF Conference, the first collection of essays showcases the invaluable perspectives and insights of some of the women who have dedicated a decade or more of their careers to advancing the NF field.

More than just a collection of narratives, “Women in NF” serves as a testament to women’s indispensable role in the effort to end NF. Spearheaded by Drs. Meena Upadhyaya and Peggy Wallace, this first set of contributions provides history but also underscores the importance of diversity and inclusion in biomedical research, patient care, and volunteer activities. We hope these essays inspire, educate, and empower others, igniting a new era of awareness and appreciation for the vital role of women in shaping the future of NF research and treatment.

The launch of this essay series is dedicated to geneticist Dr. Ludwine Messiaen in honor of her legacy of extraordinary impact in the NF field.



The first “Women in NF” essay collection is available to read now on the Children’s Tumor Foundation website at [ctf.org/womeninnf](https://ctf.org/womeninnf)



**Our thanks to the women in this collection, who are exemplary and serve as role models for future generations.**

# The Friedrich von Recklinghausen Award: Neurofibromatosis 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.



## 2024 Friedrich von Recklinghausen Award Recipient

It is with great pleasure that the Children's Tumor Foundation announces the recipient of the 2024 Friedrich von Recklinghausen Award, Rosalie Ferner, MD, consultant neurologist of Guys and St. Thomas NHS Foundation Trust London (GSTT), UK.

After an early academic career studying modern languages, Dr. Ferner shifted gears and moved on to study medicine, and hence, has spent her lifetime dedicated to improving the lives of those afflicted with neurofibromatosis and the schwannomatosis. Dr. Ferner has consistently demonstrated all the considerable attributes and accomplishments that make her a most worthy awardee.

Dr. Ferner established, and has been national lead for the nationally commissioned NF1 service in the UK since 2009 and was lead for the London NF2 service from 2010 to 2015. In these roles she has been a passionate champion for patients with all forms of neurofibromatosis and schwannomatosis but has had particular impact in the field of NF1. She is a clinician's clinician—skilled, precise, compassionate, creative and dedicated.

As a researcher, she has driven entire chapters of NF1 research in her efforts to improve clinical care for people with NF1 and has miraculously created pathways for care for people with NF in the UK that follow the best evidence in the face of many hurdles. She was instrumental in the development of QOL patient-focused outcome measures for NF1 and NF2 and has delivered multiple publications on elucidating the phenotype of NF1, OPG, and MPNST. Indeed, she is one of the most published researchers in NF still active in the field. Dr. Ferner recruits and trains early stage clinician investigators and is the consummate collaborator—expert, reliable, respectful, dedicated and clear about her mission and the importance of the work. Much of what we know about optic pathway gliomas, plexiform neurofibromas and MPNST is due to her efforts. She is driven by concern for the person facing a challenging illness and parlays this concern into critical research and national program building. Finally, no one can match her wit or sincere warmth, caring and compassion.

The Children's Tumor Foundation, along with her colleagues and peers, is proud and thrilled to recognize Dr. Ferner with the 2024 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. Ferner for this well-deserved honor.

The following are the most recent recipients of the Award:



**2023**  
Margaret (Peggy) Wallace, PhD  
University of Florida



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



**2021**  
Marco Giovannini, MD, PhD  
University of California  
Los Angeles



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

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

**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



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



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



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



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

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

Some talk titles and/or speakers may be different due to last minute changes.

# AGENDA

## Thursday · 20 June 2024 (All times are CET)

7:30	17:00	<b>REGISTRATION &amp; CHECK IN</b>	Reception
9:30	13:00	<b>Young Investigator Day – Morning Session</b> <i>Young Investigator Day is a closed meeting, by invitation only.</i> 9:30 – 13:00: Presentations, Introductions, and First Science Block 13:00 – 14:00: Informal lunch for networking and interaction	Studio
14:00	17:00	<b>REiNS 2024 Summer Meeting</b>  The Response Evaluation in Neurofibromatosis and Schwannomatosis (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 and upcoming REiNS working groups efforts. Meeting is open to all.	Auditorium
14:00	18:00	<b>Young Investigator Day – Afternoon Session</b> <i>Young Investigator Day is a closed meeting, by invitation only.</i> Mentoring Activities, Second Science Block: Poster Session	Studio
18:00	21:00	<b>Young Investigator Day – Evening Session</b> <i>Young Investigator Day is a closed meeting, by invitation only.</i> Dinner with Keynote Address and Q&A	Studio

## Friday · 21 June 2024 (All times are CET)

7:30	17:00	<b>REGISTRATION &amp; CHECK IN</b>	Reception
8:30	11:30	<b>CLINICAL CARE SYMPOSIUM</b>  Session Co-Chairs: Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital, US</i> ; Amedeo Azizi, MD, <i>Medizinische Universitaet Wien, Austria</i>	Auditorium
8:30	8:45	<b>Welcome and Introductions:</b> CCAB US/CCAB Europe Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital, US</i> ; Amedeo Azizi, MD, <i>Medizinische Universitaet Wien, Austria</i>	
8:45	9:15	<b>Topical Therapies for Cutaneous Neurofibromas</b> Carlos Romo, MD, <i>Johns Hopkins, US</i>	
9:15	9:45	<b>Clinical Considerations in Variant Analysis</b> Alicia Gomes, MS, CGS, <i>University of Alabama at Birmingham, US</i>	
9:45	10:00	<b>BREAK</b>	
10:00	10:30	<b>Breast Cancer Risk in NF Patients</b> Juha Peltonen, MD, PhD, <i>University of Turku, Finland</i>	
10:30	11:30	<b>NF2 Management</b> <b>Case Presenter and Moderator:</b> Nicole Ullrich, MD, PhD, <i>Children's Hospital Boston, US</i> <b>Discussion Panel:</b> Pediatrics: Amedeo Azizi, MD, <i>Medizinische Universitaet Wien, Austria</i> Genetic Counseling: Alicia Gomes, MS, CGC, <i>University of Alabama at Birmingham, US</i> Adult Case: Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital, US</i>	
9:00	16:00	<b>Patient Day Program</b> Patient Day Registration is open to NF patients and lay groups (patient advocates).	Studio
9:00	10:30	<b>Workshop: Requesting, Reusing, and Analyzing Data on the NF Data Portal</b> <i>Hosted by NF Open Science Initiative (NF-OSI)</i>  During this 90 minute training, attendees will receive hands-on instruction from Sage Bionetworks on how to request specific datasets and send data to analysis platforms linked to the data portal. Additionally, the Sage team will present case studies demonstrating how researchers can repurpose datasets.  This session on how to locate, request, and reuse datasets from the NF Data Portal, is tailored for users of all skill levels, from those with fundamental computer skills to experienced bioinformaticians.	Cinema

# AGENDA

Friday · 21 June 2024 (All times are CET)

10:30	12:00	<p><b>Workshop: Data Curation with Large Language Models</b>  <i>Hosted by NF Open Science Initiative (NF-OSI)</i></p> <p>This 90 minute workshop will offer participants hands-on experience in data curation, with a particular focus on utilizing large language models, like ChatGPT, to enhance the process. During the session, for example, attendees will curate data from scientific articles using the NF Tools Database. Large language models will be used to extract key information critical to NF research, including observations on animal models and cell lines.</p> <p>This session is designed for individuals across all levels of expertise who are interested in exploring data curation practices and the application of large language models in research contexts; training will utilize the NF Tools Database as a key example, and will provide comprehensive insights and opportunities for skill development.</p>	Cinema
11:30	12:15	<b>Lunch Break</b> – Lunch options will be available for purchase on site.	Riverside
12:15	13:00	<b>OPENING REMARKS</b>	Auditorium
12:15	12:30	<p><b>Honorary Co-Chairs Opening</b>            Eric Legius, MD, PhD, <i>KU Leuven, Belgium</i>            Remembering Ludwine Messiaen, PhD</p>	
12:30	12:40	<p><b>Welcome &amp; Overview: Conference Co-Chairs:</b>            Hilde Brems, PhD, <i>KU Leuven, Belgium</i>            Ignacio Blanco, MD, PhD, <i>Hospital Universitari Germans Trias i Pujol, Spain</i>            Justin Jordan, MD, PhD, <i>Massachusetts General Hospital, US</i>            Laura Klesse, MD, PhD, <i>University of Texas Southwestern Medical Center, US</i></p>	
12:40	12:45	<p><b>Introduction</b>            Annette Bakker, PhD, <i>Children's Tumor Foundation, US</i></p>	
12:45	13:00	<p><b>Opening Address</b>            Nathalie Moll, <i>Director General, EFPIA - European Federation of Pharmaceutical Industries and Associations</i></p>	
13:00	15:25	<b>GENE THERAPY</b>	Auditorium
13:00	13:10	<p>Session Co-Chairs: Elisabeth Castellanos, PhD, ErCLG, <i>Fundació Institut d'Investigació en Ciències de la Salut Germans Trias i Pujol, Spain</i>; Kathrin Meyer, PhD, <i>Alcyone Therapeutics, US</i>; Peggy Wallace, PhD, <i>University of Florida, US</i></p> <p><b>Session Chair:</b> Elisabeth Castellanos, PhD, ErCLG, <i>Fundació Institut d'Investigació en Ciències de la Salut Germans Trias i Pujol, Spain</i>, and <b>Session Co-Chair:</b> Peggy Wallace, PhD, <i>University of Florida, US</i></p>	
13:10	13:35	<p><b>Invited Speaker: RNA Therapeutic Approaches for NF1</b>            Santiago Vernia, PhD, <i>Imperial College London, UK</i></p>	
13:35	13:50	<p><b>Platform: Gene Editing Corrects an NF1 Causal Mutation and Restores Neurofibromin Expression <i>In Vitro</i></b>            Madeleine Sitton, <i>Duke University, US</i></p>	
13:50	14:05	<p><b>Platform: Preclinical Development and <i>In Vivo</i> Delivery of Antisense Oligonucleotides for Targeted NF1 Exon 17 Skipping</b>            Deeann Wallis, PhD, <i>University of Alabama at Birmingham, US</i></p>	
14:05	14:25	<b>BREAK</b>	
14:25	14:30	<b>Session Co-Chair:</b> Kathrin Meyer, PhD, <i>Alcyone Therapeutics, US</i>	
14:30	14:55	<p><b>Invited Speaker: Schwannoma Gene Therapy: AAV-Mediated Delivery of the Inflammasome Adaptor, ASC</b>            Gary Brenner, MD, PhD, <i>Massachusetts General Hospital, US</i></p>	
14:55	15:10	<p><b>Platform: Promising Adeno-Associated Viral (AAV) Gene Replacement Therapy for NF2 Effectively Reduces Tumor Growth in <i>In Vitro</i> and in <i>In Vivo</i> Schwannoma Models</b>            Krizelle Alcantara, <i>Nationwide Children's Hospital, US</i></p>	
15:10	15:25	<p><b>Platform: Testing the Potential of Antisense Gene Therapy for NF2-Related Schwannomatosis in an iPSC-Based Model</b>            Gemma Casals Sendra, <i>Germans Trias i Pujol Research Institute, Spain</i></p>	

# AGENDA

## Friday · 21 June 2024 (All times are CET)

15:25	16:30	<b>KEYNOTE #1: Gene Therapy of Metachromatic Leukodystrophy from Bench to Bedside and the Market: A Roadmap to the Development of New Advanced Gene and Cell Therapies</b> Luigi Naldini, MD, PhD, <i>San Raffaele University, Italy</i>	Auditorium
16:30	16:45	<b>Session Perspective: Gene Therapy</b> Session Co-Chairs will give their perspective on Gene Therapy.	Auditorium
16:30	16:45	<b>Perspectives:</b> Final Conclusions and Discussion: Future Perspectives for Gene Therapy in NF1 and Schwannomatosis	
16:45	17:45	<b>Presentation of Awards</b> <b>Theodor Schwann Awards</b> (Basic & Clinical): Presented by Rianne Oostenbrink, <i>European NF Group</i> <b>Friedrich von Recklinghausen Award:</b> Presented by Annette Bakker, <i>Children's Tumor Foundation</i> Remarks from NF patients, Monique Aerts and Amit Ghose Remarks from our Platinum Sponsors: Alexion AstraZeneca Rare Disease and SpringWorks Therapeutics A champagne toast to our Award recipients!	Auditorium
18:30	21:30	<b>Welcome Reception</b> The 2024 Global NF Conference Welcome Reception will be held on Friday evening, 21 June 2024, at the beautiful Royal Museums of Fine Arts of Belgium. This reception is planned as a 'walking dinner', with continuous hors d'oeuvres and buffet stations, and access to view the exhibitions throughout the museum for lots of relaxed networking and socializing. This is a ticketed event and space is limited.	Royal Museums of Fine Arts of Belgium

## Saturday · 22 June 2024 (All times are CET)

7:00	17:00	<b>REGISTRATION &amp; CHECK IN</b>	Reception
7:15	8:45	<b>Navigating Transitions of Care in Patients with NF1-PN: A Case-Based Discussion</b> <i>*This is an independent educational satellite symposium hosted by SpringWorks Therapeutics. It will not be live-streamed or available for on-demand viewing on the conference app.*</i> The intricacies of navigating NF1-PN patient care in a range of different healthcare environments can pose a significant burden to patients, their caregivers, and health care providers. Hear experts explore strategies for effective transition and continuity of care from childhood to adulthood for patients with NF1-PN.	Studio
9:00	10:00	<b>KEYNOTE #2: The Science and the Art of Resilience: 5 Lessons Learned from Patients, Communities, and Society</b> Abby Rosenberg, MD, <i>Boston Children's Hospital / Harvard University, US</i>	Auditorium
10:15	10:30	<b>Session Perspective: Comprehensive Care</b> Session Co-Chairs will give their perspective on Comprehensive Care.	Auditorium
10:15	10:30	<b>Perspectives:</b> Eric Legius, MD, PhD, <i>KU Leuven, Belgium</i> , will provide the Co-Chair perspective	
10:30	12:00	<b>COMPREHENSIVE CARE – Morning Session</b> Session Co-Chairs: Miriam Bornhorst, MD, <i>Children's National Hospital, US</i> ; Eric Legius, MD, PhD, <i>KU Leuven, Belgium</i> ; Karin Walsh, PsyD, <i>Children's National Hospital, US</i>	Auditorium
10:30	11:00	<b>Invited Speaker: Understanding Metabolism in Patients with NF1</b> Miriam Bornhorst, MD, <i>Children's National Hospital, US</i>	

# AGENDA

Saturday · 22 June 2024 (All times are CET)

11:00	11:15	<b>Platform: Exploring the Interplay Between Lipid Metabolism and LZTR1 in Peripheral Nerve Pathologies</b> Georgia Daraki, Leibniz Institute on Aging-Fritz Lipmann Institute, Germany	
11:15	11:30	<b>Platform: Disruption of Core Clock Gene Expression in NF1-Associated Schwann Cells</b> Anja Harder, MD, PhD, University Medical Centre of the Johannes Gutenberg University Mainz, Germany	
11:30	11:45	<b>Platform: Abnormal Circadian Excretion of 6-Sulfatoxymelatonin in Women with NF1</b> Maria Isis Atallah Gonzalez, MD, PhD, Lausanne University Hospital, Switzerland	
11:45	12:00	<b>Platform: Age Related Change in the Magnetic Resonance Imaging Characteristics of Plexiform Neurofibromas in Patients with Neurofibromatosis Type 1</b> Eva Dombi, MD, National Cancer Institute, US	
<b>12:00</b>	<b>13:00</b>	<b>Lunch Break</b> – Lunch options will be available for purchase on site.	Riverside
<b>13:00</b>	<b>14:30</b>	<b>COMPREHENSIVE CARE – Afternoon Session</b> Session Co-Chairs: Miriam Bornhorst, MD, Children’s National Hospital, US; Eric Legius, MD, PhD, KU Leuven, Belgium; Karin Walsh, PsyD, Children’s National Hospital, US	Auditorium
13:00	13:30	<b>Invited Speaker: Examining the Relation Between Learning and Attention Deficits and Sleep Disturbance in Children with Neurofibromatosis Type 1 (NF1)</b> Karin Walsh, PsyD, Children’s National Hospital, US	
13:30	13:45	<b>Platform: Comparative Analysis of Validated Measures of Cognitive, Behavioral, Motor, and Physiological Impairments in a Pediatric Population of Neurofibromatosis Type 1</b> Lindsey Aschbacher-Smith, Cincinnati Children’s Hospital Medical Center, US	
13:45	14:15	<b>Invited Speaker: Transition from Paediatric to Adult Care NF1, NF2 Schwannomatosis &amp; Non NF2 Schwannomatosis</b> Rosalie Ferner, Guys and St. Thomas’ NHS Foundation Trust, UK	
14:15	14:30	<b>Platform: Supporting Reproductive Choices and Decision-Making for Adults with Neurofibromatosis</b> Jane Fleming, PhD, Royal North Shore Hospital, Australia	
<b>14:30</b>	<b>15:30</b>	<b>CONSORTIA UPDATES</b>	Auditorium
14:30	14:50	<b>Neurofibromatosis Clinical Trials Consortium</b> Michael Fisher, MD, Children’s Hospital of Philadelphia, US	
14:50	15:10	<b>Optic Pathway Glioma Consortium “An International Multi-Center Natural History Study of Newly Diagnosed NF1- Associated Optic Pathway Glioma (NF1-OPG)”</b> Robert Avery, DO, MSCE, Children’s Hospital of Philadelphia, US	
15:10	15:30	<b>Global Guidelines for Care</b> Scott Plotkin, MD, PhD, Massachusetts General Hospital, US	
<b>15:30</b>	<b>17:30</b>	<b>Combined Poster Sessions &amp; Cocktail Reception</b> Combined Basic and Clinical Science Poster Session. Refreshments to be provided.	Grand Hall
<b>17:30</b>	<b>20:30</b>	<b>cNF Satellite Meeting: Translational and Clinical Advances for Cutaneous Neurofibroma</b> Hosted by Neurofibromatosis Therapeutic Acceleration Program (NTAP)	Studio
17:30	17:40	<b>Introduction</b> Jaishri Blakeley, MD, Johns Hopkins University, US	
17:40		<b>cNF Biology and Therapeutic Discovery</b> Moderator: Pierre Wolkenstein, MD, PhD, Paris-Est Créteil University, France	
17:40	17:50	<b>A Fly Approach to Pathway and Drug Discovery for Cutaneous Neurofibromas</b> Ross Cagan, PhD, University of Glasgow, Scotland	
17:50	18:00	<b>In Vivo Testing of Drugs Targeting Non-MAPK Pathways to Regress cNFs in NF1</b> Piotr Topilko, PhD, Mondor Institute for Biomedical Research, France	
18:00		<b>Measuring cNF Burden in cNF Trials</b> Moderator: Yemima Berman, BMBS, BSc Hons, PhD, FRACP, The University of Sydney, Australia	
18:00	18:10	<b>Comparison of Camera Devices</b> Jane Fleming, PhD, Northern Sydney LHD, Australia	



# AGENDA

## Saturday · 22 June 2024 (All times are CET)

18:10	18:20	<b>Outcome Domain and Minimum Dataset</b> Laura Fertitta, MD, <i>Henri Mondor Hospital, CERENEF, France</i>
18:20	18:30	<b>Portable Tools to Quantify Stiffness as Outcome Measure for Cutaneous Neurofibroma</b> Lu Le, MD, PhD, <i>University of Virginia School of Medicine</i>
18:30		<b>cNF Clinical Trials</b> Moderator: Jaishri Blakeley, MD, <i>Johns Hopkins University, US</i>
18:30	18:40	<b>Mirdametinib</b> Carlos Romo, MD, <i>Johns Hopkins University, US</i>
18:40	18:50	<b>NFlection</b> Kavita Sarin, MD, PhD, <i>Stanford University, US</i>
18:50	19:00	<b>MGH Device and Local Therapy</b> Patricia Richey, MD, <i>Massachusetts General Hospital, US</i>
19:00	19:20	<b>BREAK</b> Beer, Cheese and Chocolate
19:20	20:20	<b>Panel Discussion</b> Moderator: Carlos Romo, MD, <i>Johns Hopkins University, US</i>  <b>Is MEKi the Right Mechanism for Treating cNF?</b> Panelists: Eduard Serra, PhD, <i>Germans Trias i Pujol Research Institute, Spain</i> ; Harish Vasudevan, MD, PhD, <i>University of California San Francisco, US</i>  <b>What is the Best Measurement for cNF Outcome?</b> Panelists: Vanessa Merker, PhD, <i>Massachusetts General Hospital, US</i> ; Yemima Berman, BMBS, BSc Hons, PhD, <i>The University of Sydney, Australia</i> ; Justin Jordan, MD, MPH, <i>Massachusetts General Hospital, US</i>
20:20	20:30	<b>Closing Remarks</b> Annette Bakker, <i>Children's Tumor Foundation, US</i>

## Sunday · 23 June 2024 (All times are CET)

<b>7:00</b>	<b>17:00</b>	<b>REGISTRATION &amp; CHECK IN</b>	Reception
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### Caregiver and Healthcare Professional Perspectives on the Continuity of Care in Paediatric, Symptomatic, Inoperable NF1-PN

*\*This is an independent educational satellite symposium hosted by Alexion, AstraZeneca Rare Disease. It will not be live-streamed or available for on-demand viewing on the conference app.\**

<b>7:15</b>	<b>8:45</b>	Join us for an interview of a caregiver, who will share their experience caring for a paediatric patient with symptomatic, inoperable NF1-PN.  The objectives of this symposium are to: <ul style="list-style-type: none"> <li>• Present long-term data from the SPRINT clinical trial and discuss real-world experiences in paediatric patients with symptomatic, inoperable NF1-PN</li> <li>• Explore the outcomes of continued treatment and the importance of disease management</li> </ul>	Studio
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<b>9:00</b>	<b>10:00</b>	<b>KEYNOTE #3: Update on the Diagnosis and Management of Chronic Neuropathic Pain</b> Andrew Rice, MD, <i>Imperial College London, UK</i>	Auditorium
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<b>10:15</b>	<b>10:30</b>	<b>Session Perspective: Schwannomatosis</b> Session Co-Chairs will provide their perspective on Schwannomatosis.	Auditorium
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10:15	10:30	<b>Perspectives:</b> Gareth Evans, MD, <i>University of Manchester, UK</i> , will provide the Co-Chair perspective	
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<b>10:30</b>	<b>12:05</b>	<b>SCHWANNOMATOSIS – Morning Session</b> Session Co-Chairs: Gareth Evans, MD, <i>University of Manchester, UK</i> ; Liyam Laraba, PhD, <i>University of Plymouth, UK</i> ; Masahiro Toda, MD, PhD, <i>Keio University, Japan</i>	Auditorium
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10:30	10:55	<b>Invited Speaker: DGCR8 and Schwannomatosis: Not Dicing Within the 22q</b> Barbara Rivera, PhD, <i>McGill University, Canada</i>	
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# AGENDA

Sunday · 23 June 2024 (All times are CET)

10:55	11:10	<b>Platform:</b> Characterization of the NF2-Associated Immunoepitome Joe Kissil, PhD, <i>H. Lee Moffitt Cancer Center, US</i>	
11:10	11:25	<b>Platform:</b> LZTR1 and SMARCB1 Mutations in Schwann Cells Induce Increased Pain Sensitivity Through Distinct Mechanisms Larry Sherman, PhD, <i>Oregon Health &amp; Science University, US</i>	
11:25	11:40	<b>Platform:</b> NF2-Related Schwannomatosis: An Updated Genetic and Epidemiological Study Gareth Evans, MD, <i>University of Manchester, UK</i>	
11:40	12:05	<b>Invited Speaker:</b> Targeting the Schwannoma-Neuron-Macrophage Crosstalk for the Treatment of Schwannomatosis and Associated Pain Lei Xu, MD, PhD, <i>Harvard Medical School, US</i>	
<b>12:05</b>	<b>13:00</b>	<b>Lunch Break</b> – Lunch options will be available for purchase on site.	Riverside
<b>13:00</b>	<b>15:00</b>	<b>SCHWANNOMATOSIS – Afternoon Session</b>	Auditorium
		Session Co-Chairs: Gareth Evans, MD, <i>University of Manchester, UK</i> ; Liyam Laraba, PhD, <i>University of Plymouth, UK</i> ; Masahiro Toda, MD, PhD, <i>Keio University, Japan</i>	
13:00	13:25	<b>Invited Speaker:</b> Sensitivity of Pathogenic Variant Detection in Schwannomatosis Miriam Smith, PhD, <i>University of Manchester, UK</i>	
13:25	13:40	<b>Platform:</b> Effect of Bevacizumab on Intracranial Non-Target Meningiomas and Non-Vestibular Schwannomas in Persons with NF2-Related Schwannomatosis: An NF Clinical Trials Consortium Study (NF104) Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital, US</i>	
13:40	13:55	<b>BREAK</b>	
13:55	14:20	<b>Invited Speaker:</b> CDKN2A in Meningioma Gelareh Zadeh, MD, PhD, FRCSC, <i>University of Toronto, Canada</i>	
14:20	14:45	<b>Invited Speaker:</b> Repurposing Anti-Retroviral Drugs to Treat NF2-Related Tumours Sylwia Ammoun, PhD, <i>University of Plymouth, UK</i>	
14:45	15:00	<b>Platform:</b> Characterization of a Novel <i>In Vitro</i> Model to Study Disease Mechanisms in NF2-Related Schwannomatosis and Testing Novel Therapeutics Pipasha Biswas, MS, <i>Nationwide Children's Hospital, US</i>	
<b>15:00</b>	<b>17:00</b>	<b>BASIC / PRECLINICAL PLATFORM SESSION</b>	Auditorium
		Session Co-Chairs: Sheila Mansouri, PhD, <i>University Health Network, Canada</i> ; Harish Vasudevan, MD, PhD, <i>University of California, San Francisco, US</i>	
15:00	15:15	<b>Platform:</b> Alternatively Activated Macrophages are Associated with Faster Growth Rate in Vestibular Schwannoma Grace Gregory, BSc, <i>University of Manchester, UK</i>	
15:15	15:30	<b>Platform:</b> Identification of Synthetic Lethality Targets through Genome-Wide CRISPR Screen in NF1- and NF1/SUZ12-Deficient Human Schwann Cells and MPNST Cell Lines Julia Nikrad, PhD, <i>University of Minnesota, US</i>	
15:30	15:45	<b>Platform:</b> Targeting Granulocyte-Macrophage Colony Stimulating Factor Signaling in Plexiform Neurofibroma Jay Pundavela, PhD, <i>Cincinnati Children's Hospital Medical Center, US</i>	
15:45	16:00	<b>Platform:</b> A Platform for Rapid NF1 Patient-Derived Benign and Malignant Tumor Organoid Establishment and Screening Alice Soragni, PhD, <i>University of California, Los Angeles, US</i>	
16:00	16:15	<b>Platform:</b> Towards an iPSC-Based MPNST Model: Impact of NF1, CDKN2A and SUZ12 Inactivation in Cell Function and Identity Itziar Uriarte-Arrazola, MSc, <i>Germans Trias i Pujol Research Institute, Spain</i>	
16:15	16:30	<b>Platform:</b> Targeting Tumor Associated Macrophages to Fight MPNST Francesca Scantamburlo, MSc, <i>University of Padova, Italy</i>	
16:30	16:45	<b>Platform:</b> Intratumoral Plasma Cells Are Required for a Durable Response to Anti-PDL1 Therapy in <i>De Novo</i> MPNSTs Joshua Lingo, BS, <i>University of Iowa, US</i>	
16:45	17:00	<b>Platform:</b> Presentation by Young Investigator Day participant to be announced on 6/20.	

# AGENDA

Monday · 24 June 2024 (All times are CET)

7:30	17:00	<b>REGISTRATION &amp; CHECK IN</b>	Reception
8:00	9:00	<b>LATE BREAKING ABSTRACTS</b>	Auditorium
8:00	8:15	<b>Platform:</b> Persistence of Radial Glia Cells After Differentiation of NF2-Mutant Human Neuroepithelial Stem (NES) Cells Jignesh Tailor, BMBCh, PhD, FRCSC, <i>Indiana University School of Medicine, US</i>	
8:15	8:30	<b>Platform:</b> RASopathies Influences on Neuroanatomical Variation in Children Tamar Green, MD, <i>Stanford Medicine, US</i>	
8:30	8:45	<b>Platform:</b> Trametinib in NF1-Related Plexiform Neurofibromas: Preliminary Results of One Year Follow-Up Christine Noordhoek, <i>Erasmus Medical Center, Netherlands</i>	
8:45	9:00	<b>Platform:</b> Patient-Reported Outcomes of Pain Severity and Pain Interference from ReNeu: Pivotal Phase 2b Trial of Mirdametinib in Adults and Children with Neurofibromatosis Type 1-Associated Plexiform Neurofibroma (NF1 PN) Dusica Babovic-Vuksanovic, <i>Mayo Clinic, US</i>	
9:00	10:00	<b>KEYNOTE #4: Reimagining Druggability Using Chemoproteomic Platforms</b> Daniel Nomura, PhD, <i>Univeristy of California, Berkley, US</i>	Auditorium
10:10	10:15	<b>Session Perspective: Novel Therapeutics</b>	Auditorium
		Session Co-Chairs will give their perspective on Novel Therapeutics.	
10:10	10:15	<b>Perspectives:</b> Amedeo Azizi, MD, <i>Medizinische Universitaet Wien, Austria</i> , will provide the Co-Chair perspective	
10:15	12:00	<b>NOVEL THERAPEUTICS – Morning Session</b>	Auditorium
		Session Co-Chairs: Amedeo Azizi, MD, <i>Medizinische Universitaet Wien, Austria</i> ; Wade Clapp, MD, <i>Indiana University, US</i> ; Laura Ferritta, MD, <i>Henri Mondor Hospital, CERENEF, France</i>	
10:15	10:45	<b>Invited Speaker: Targeting Schwann Cell-Tumor Microenvironment Interactions in NF1</b> Lu Le, MD, PhD, <i>University of Virginia School of Medicine, US</i>	
10:45	11:00	<b>Platform:</b> Proteomics Reveals Potential Therapeutic Targets for Malignant Peripheral Nerve Sheath Tumors Based on Chr8q Status Belinda Garana, PhD, <i>Pacific Northwest National Laboratory, US</i>	
11:00	11:15	<b>Platform:</b> Personalized MPNST Pre-Clinical Testing Using PDOX: Providing Treatment Possibilities to a Molecular Tumor Board Sara Ortega-Bertran, <i>Hereditary Cancer Program, Catalan Institute of Oncology (ICO-IDIBELL), Spain</i>	
11:15	11:30	<b>Platform:</b> Combined Efficacy of SOS1 and KRASmulti Inhibitors in Malignant Peripheral Nerve Sheath Tumors Özlem Yüce Petronczki, PhD, <i>Böhringer Ingelheim RCV GmbH &amp; Co, Vienna, Austria</i>	
11:30	11:45	<b>Platform:</b> Vertical Inhibition of ERK Signalling is Effective in Preclinical Models of Malignant Peripheral Nerve Sheath Tumors Jiawan Wang, PhD, <i>Johns Hopkins University School of Medicine, US</i>	
11:45	12:00	<b>Platform:</b> Combined MEK and Histone Deacetylase Inhibition Exploits a Targetable Vulnerability in Polycomb Repressor Complex 2 Deficient Malignant Peripheral Nerve Sheath Tumors Kyle Williams, PhD, <i>University of Minnesota, US</i>	
12:00	12:55	<b>Optional Lunch Satellite: VCEP</b>	Studio
		Overview and updates from the International ClinGen NF1/SWN Variant Curation Expert Panel (VCEP)	
12:05	12:25	<b>What is the NF1/SWN VCEP and How Will it Help My Patients?</b> Alicia Gomes, MS, CGC, <i>University of Alabama, Birmingham, US</i>	

# AGENDA

Monday · 24 June 2024 (All times are CET)

12:25 12:40 **Case Presentation of a Patient with Café-au-Lait Macules and an NF1 Variant of Uncertain Significance (VUS)**  
Magdalena Koczkowska, PhD, *Medical University of Gdansk, Poland*

12:40 12:55 **Reclassification of an NF1 Variant Initially Identified in a Patient with NF1**  
Kara Anstett, MS, CGC, *New York University Langone Health, US*

**13:00 15:00 NOVEL THERAPEUTICS – Afternoon Session** Auditorium

Session Co-Chairs: Amedeo Azizi, MD, *Medizinische Universitaet Wien, Austria*;  
Wade Clapp, MD, *Indiana University, US*; Laura Ferritta, MD, *Henri Mondor Hospital, CERENEF, France*

13:00 13:25 **Invited Speaker: Exploring Novel Therapeutics in Children – Lessons Learned from Pediatric Oncology**  
Darren Hargrave, MD, *UCL Great Ormond Street Institute of Child Health, UK*

13:25 13:40 **Platform: MEK/SHP2 Inhibition Prevents Congenital Pseudarthrosis of the Tibia Caused by NF1 Loss in Schwann Cells and Skeletal Stem/Progenitor Cells**  
Celine Colnot, PhD, *INSERM, Mondor Biomedical Research Institute, Paris-Est Creteil University, France*

13:40 13:55 **Platform: A Randomized Double-Blind, Vehicle-Controlled, Phase 2b Study of NF1-179 (Nedometinib) Topical Gel for the Treatment of Cutaneous Neurofibromas in Neurofibromatosis Type 1**  
Kavita Sarin, MD, PhD, *Stanford University, US*

13:55 14:05 **BREAK**

14:05 14:20 **Platform: Targeted Exon Skipping of NF1 Exon 52 as a Mutation-Specific Therapeutic for Neurofibromatosis Type 1**  
Cameron Church, *University of Alabama at Birmingham, US*

14:20 14:35 **Platform: Targeting the Tumor Microenvironment to Improve Immunotherapy**  
Lei Xu, MD, PhD, *Massachusetts General Hospital, US*

14:35 15:00 **Invited Speaker: Next Gen Clinical Trials for NF1 and Schwannomatosis: What's on the Horizon**  
Scott Plotkin, MD, PhD, *Massachusetts General Hospital, US*

**15:00 17:00 CLINICAL PLATFORM SESSION** Auditorium

Session Co-Chairs: Katrina Morris, MD, PhD, *Concord Repatriation General Hospital, Australia*; Thorsten Rosenbaum, MD, PhD, *Sana Hospital Duisburg, Germany*

15:00 15:15 **Platform: Clinical Features Predictive of Plexiform Neurofibroma Response to Mitogen-Activated Kinase Inhibitor**  
Chelsea Kotch, MD, MSCE, *Children's Hospital of Philadelphia, US*

15:15 15:30 **Platform: Genetic Influence, Individual Genetic Profiling and Targetable Treatment in Patients with NF2-Associated Vestibular Schwannomas**  
Isabel Gugel, MD, PhD, *University Hospital Tübingen, Germany*

15:30 15:45 **Platform: ReNeu: A Pivotal Phase 2b Trial of Mirdametinib in Children and Adults with Neurofibromatosis Type 1 (NF1)-Associated Symptomatic Inoperable Plexiform Neurofibroma (PN)**  
Christopher Moertel, MD, *University of Minnesota School of Medicine, US*

15:45 16:00 **Platform: Demographic and Disease Predictors of Executive Functions in Children and Adolescents with Neurofibromatosis Type 1: Results from the Largest International Dataset**  
Xiaoli Zong, PhD, *Florida State University College of Medicine, US*

16:00 16:15 **Platform: Characteristics and Association of Pain and Quality of Life at Baseline and with Treatment in Children with Neurofibromatosis Type 1 and Plexiform Neurofibromas Enrolled on a Phase II Trial of Selumetinib**  
Pamela Wolters, PhD, *National Cancer Institute, US*

16:15 16:30 **Platform: Non-Invasive High Intensity Focused Ultrasound (HIFU) Treatment of Cutaneous Neurofibromas**  
Sirrku Peltonen, PhD, *University of Helsinki and Helsinki University Hospital, Finland*

# AGENDA

## Monday · 24 June 2024 (All times are CET)

16:30	16:45	<b>Platform: Overcoming Barriers to Breast Screening in NF1</b> Yemima Berman, BMBS FRACP BSc Hons PhD, <i>Royal North Shore Hospital, Australia</i>	
16:45	17:00	<b>Platform: Incidence of Ophthalmic Complications in NF-1 Patients Treated with MEK Inhibitors</b> Zsila Sadighi, MD, <i>University of Texas MD Anderson Cancer Center, US</i>	
17:00	17:15	<b>Platform: Predicting the Clinical Phenotype in NF2-Related Schwannomatosis Patients</b> Marcia Eoli, MD, <i>Fondazione IRCCS Istituto Besta, Italy</i>	

### Fireside Chat

Introduction: Annette Bakker, *Children's Tumor Foundation, US*

<b>17:30</b>	<b>18:30</b>	A fireside chat between <b>Dr. Niklas Blomberg</b> , Executive Director of Innovative Health Initiative, and <b>Magda Chlebus</b> , Executive Director Scientific & Regulatory Affairs at EFPIA and board member of CTF Europe, to discuss the unique public-private partnership and other rare disease opportunities that Europe holds. Light refreshments will be served.	Studio
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## Tuesday · 25 June 2024 (All times are CET)

<b>7:30</b>	<b>13:00</b>	<b>REGISTRATION &amp; CHECK IN</b>	Reception
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<b>8:00</b>	<b>9:00</b>	<b>Poster Competition Winners – Oral Presentations</b> Each winner of the Poster Competition will give a platform presentation with time for Q&A.	Auditorium
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<b>9:00</b>	<b>10:00</b>	<b>KEYNOTE #5: Engineering Serendipity: AI's Rapidly Expanding Role in Research and Care</b> Casey Greene, PhD, <i>University of Colorado</i>	Auditorium
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<b>10:15</b>	<b>10:30</b>	<b>Session Perspective: AI, Novel Technologies, Biomarkers</b> Session Co-Chairs will give their perspective on AI, Novel Technologies, and Biomarkers.	Auditorium
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10:15	10:30	<b>Perspectives: TBA</b>	
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<b>10:30</b>	<b>12:00</b>	<b>AI, NOVEL TECHNOLOGIES, BIOMARKERS – Morning Session</b> Session Co-Chairs: Said Farschtschi, MD, <i>University Medical Center Hamburg-Eppendorf, Germany</i> ; Eduard Serra, PhD, <i>Germans Trias i Pujol Research Institute, Spain</i>	Auditorium
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10:30	10:45	<b>Invited Speaker: A Multicenter Radiomics Model for Diagnosis of NF1-Associated Peripheral Nerve Sheath Tumors</b> Scott Plotkin, MD, PhD, <i>Massachusetts General Hospital, US</i>	
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10:45	11:00	<b>Invited Speaker: Automatic Detection and Differentiation of Neurofibromas in NF1 – Current Status of Radiomics- and Deep Learning-Based Applications for MRI Image Analysis</b> Inka Ristow, MD, MHBA, <i>Medical Center Hamburg-Eppendorf (UKE), Germany</i>	
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11:00	11:15	<b>Discussion and Q&amp;A</b>	
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11:15	11:30	<b>Platform: Machine Learning and High-Content Imaging for Modeling Neurofibromin in Schwann Cells</b> Gregory Way, <i>University of Colorado School of Medicine, US</i>	
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11:30	11:45	<b>Platform: Leveraging State-Of-The-Art Model Systems to Enable AI Drug Response Prediction Algorithms in NF1 Tumors</b> Sara Gosline, <i>Senior Scientist, Pacific Northwest National Laboratory, US</i>	
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11:45	12:00	<b>Platform: Localized Magnetic Resonance Imaging Features of the Anterior Visual Pathway are Associated with Visual Acuity Loss in Children with NF1-OPG</b> Zhifan Jiang, PhD, <i>Children's National Hospital, US</i>	
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<b>12:00</b>	<b>13:00</b>	<b>Lunch Break</b> – Lunch options will be available for purchase on site.	Riverside
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# AGENDA

Tuesday · 25 June 2024 (All times are CET)

<b>13:00</b>	<b>14:45</b>	<b>AI, NOVEL TECHNOLOGIES, BIOMARKERS – Afternoon Session</b>	Auditorium
		Session Co-Chairs: Said Farschtschi, MD, <i>University Medical Center Hamburg-Eppendorf, Germany</i> ; Eduard Serra, PhD, <i>Germans Trias i Pujol Research Institute, Spain</i>	
13:00	13:30	<b>Invited Speaker: Exploration of Exosomes as Blood-Derived Biomarkers and Therapy Indicators for NF2-SWN</b> Lars Riecken, MD, <i>Leibniz Institute</i>	
13:30	13:45	<b>Platform: Multi-Omics Analysis of Multiple Meningiomas in NF2-Related Schwannomatosis</b> Yu Teranishi, MD, PhD, <i>Paris Brain Institute, France</i>	
13:45	14:00	<b>Platform: The PNF-ANF-MPNST Progression at Single Cell Level: Delving Deep into NF1-Related Tumors</b> Bernat Gel, PhD, <i>Hereditary Cancer Group, Germans Trias i Pujol Research Institute (IGTP), Spain</i>	
14:15	14:30	<b>Platform: A Comprehensive Algorithm to Predict Malignant Transformation of NF1 Nerve Sheath Tumors From Single-Cell Transcriptomic Profiling</b> Xiyuan Zhang, PhD, <i>National Institute of Health, US</i>	
14:30	14:45	<b>Platform: Epigenetic Profiling Improves Diagnostic Accuracy of Malignant Peripheral Nerve Sheath Tumors (MPNST)</b> Catena Kresbach, MD, <i>Institute of Neuropathology, University Hospital Hamburg-Eppendorf, Hamburg, Germany</i> and <i>Forschungsinstitut Kinderkrebs-Zentrum Hamburg, Germany</i>	
<b>14:45</b>	<b>15:00</b>	<b>ADJOURNMENT</b> <b>Speakers:</b> 2024 NF Conference Co-Chairs: Hilde Brems, PhD, <i>KU Leuven, Belgium</i> ; Ignacio Blanco, MD, PhD, <i>Hospital Universitari Germans Trias i Pujol, Spain</i> ; Justin Jordan, MD, PhD, <i>Massachusetts General Hospital, US</i> ; Laura Klesse, MD, PhD, <i>University of Texas Southwestern Medical Center, US</i>	Auditorium

Some talk titles and/or speakers may be different due to last minute changes.



YOUNG  
INVESTIGATOR  
DAY

NF CONFERENCE

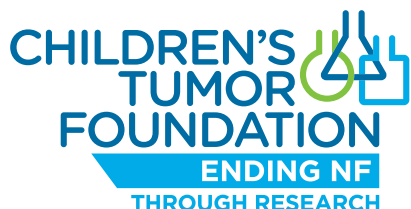
Children's Tumor Foundation is thrilled to host the inaugural Young Investigator Day (YI Day) on Thursday, June 20, 2024, in conjunction with the 2024 Global NF Conference.

Developing young investigators and supporting their growth into careers as independent NF researchers is a central priority of the Children's Tumor Foundation.

We are excited to share a day dedicated to supporting those up-and-coming researchers in networking, science, mentorship, and furthering their independent research and future career plans.

To view the Young Investigator Day program book,  
scan this code with your mobile phone.





**PATIENT DAY**

**NF CONFERENCE**



## Engaging patients in the mission to **end NF**

The Children's Tumor Foundation is passionate about engaging patients in the mission to end NF by including their input throughout the drug development and research process.

This year, we are holding our second Patient Engagement Day adjacent to our annual NF Conference to connect patients with researchers and clinicians who collectively work together to advance the NF field.

This day will include educational sessions around diagnostics, gene therapy, and the roles of patients in clinical trials - from the bench to bedside. In addition, the afternoon will be focused on collaborative discussions to identify key areas of focus in the coming year and brainstorm solutions for our biggest challenges.

Captioning in English and audio translation into several languages will be available.

**Friday,**  
**June 21, 2024**  
**from 9am - 4pm**  
The EGG  
Brussels, Belgium

***Optional social events  
on Thursday and Friday  
evenings.***

Learn more and  
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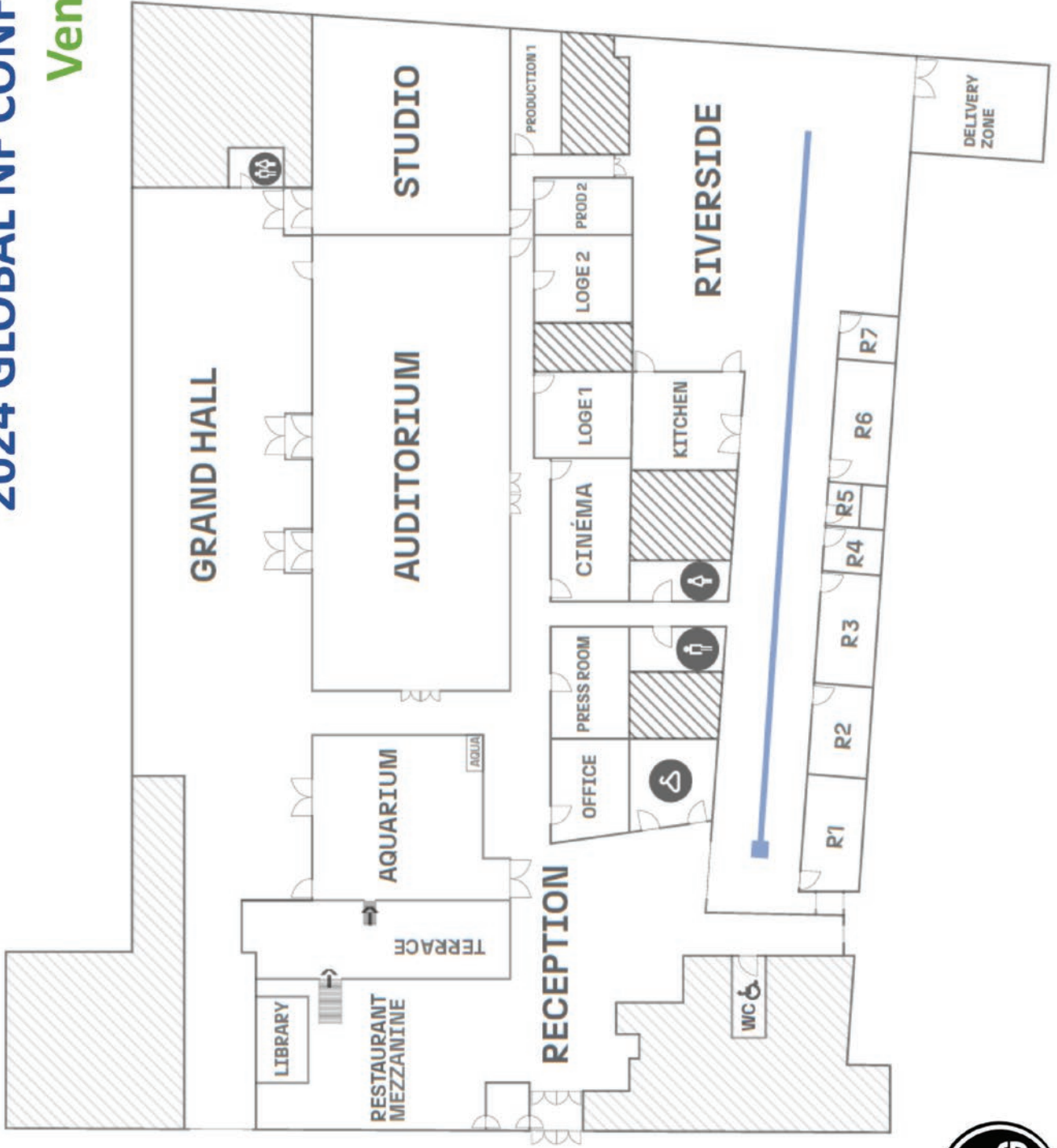
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**ALEXION**<sup>®</sup>  
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# 2024 GLOBAL NF CONFERENCE

## Venue Map



Rue Bara 175  
1070 Brussels, BE



2025  
NF CONFERENCE  
JUN 21-24 [nfconference.org](https://nfconference.org)

Omni Shoreham Hotel  
2500 Calvert St., NW  
Washington, DC

SAVE THE DATE

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