



PROGRAM (as of October 2018)

FRIDAY - 2 NOVEMBER 2018

- 08:30 - 12:30** **OPTIONAL SATELLITE SYMPOSIUM**
Developing Therapeutics for Cutaneous Neurofibromas **(PETIT AMPHI)**
Sponsored by the Neurofibromatosis Therapeutics Acceleration Program (NTAP)
- 08:30** Welcome and Overview
2016-2018 : The Changing cNF Landscape (Jaishri Blakeley, MD)
- 08:40 - 09:40** Learning from cutaneous neurofibroma models
- Piotr Topilko, PhD – Mouse models of disease
 - Lu Le, MD, PhD – Cells of origin
 - Jill Weimer, PhD – Porcine models of disease
 - Alice Soragni, PhD - 3D organoid models development
- 09:40 - 10:15** Cellular and genetic targets in cutaneous neurofibromas
- David Gutmann, MD, PhD – iPS identified genetic targets
 - Nicolas Ortonne, MD, PhD – Immunologic targets
 - Robert Allaway, PhD – Human network analysis
- 10:15 - 10:50** Assessing cutaneous neurofibromas
- Anat Stemmer-Rachamimov, MD, Pierre Wolkenstein, MD, PhD - Classification of cNF
 - Ashley Cannon, PhD, MGC – Natural history of cNF
 - Dominique Pichard, MD - Global assessment and severity scales
- 10:50 - 11:05** Break
- 11:05 - 12:05** Panel discussion with moderated Q/A (Pierre Wolkenstein)
- Basic science
 - Animal models
 - Clinical trials
 - EMA
 - Patient perspective
- 12:05 - 12:15** Final comments and adjourn

13:30 - 15:30	ANIMAL MODELS (Part 1) - BASIC Pathology of Genetically Engineered NF Animal Models: Consensus Report Co-chairs: <ul style="list-style-type: none"> • Marco Giovannini, MD, PhD, UCLA, US • Nancy Ratner, PhD, Cincinnati Children's Hospital, US • Anat-Stemmer Rachamimov, MD, Harvard University, US 	(ROOM 201)
13:30 - 15:30	EDUCATIONAL SYMPOSIUM (Part 1) - CLINICAL Co-chairs: <ul style="list-style-type: none"> • Dusica Babovic-Vuksanovic, MD, Mayo Clinic, US • Mimi Berman, MD, University of Melbourne, Australia • Karin Cunha, PhD, Federal University of Brazil <p>Diagnostic challenges</p>	(GRAND AMPHI)
13:30 - 13:55	A review of the complex and unique testing approaches available for the neurofibromatoses Alicia Gomes, MS, CGC University of Alabama at Birmingham, US	
13:55 - 14:20	Clinical overlap with RASopathies Karen Gripp, MD Nemours/Alfred DuPont Hospital for Children, US	
14:20 - 14:45	Do not forget constitutional mismatch repair deficiency (CMMRD) Katharina Wimmer, PhD, Genetics Clinic, University of Innsbruck, Austria	
14:45 - 15:20	Prenatal diagnostics: from CVS and amniocentesis to PGD Myra Wick, MD, PhD, Mayo Clinic, US; Kristi Jones, PhD, University of Sydney, Children's Hospital at Westmead, Australia	
15:20 - 15:45	Less well known dermatological manifestations in NF1 Sirrku Peltonen, PhD, University of Turku, Finland	
15:45 - 16:10	Break	
16:10 - 18:15	EDUCATIONAL SYMPOSIUM (Part 2) Co-chairs: <ul style="list-style-type: none"> • Dusica Babovic-Vuksanovic, MD • Mimi Berman, MD • Karin Cunha, PhD <p>Management challenges</p>	(GRAND AMPHI)
16:10 - 16:35	A holistic model to discuss the impact of muscular phenotype on lifestyle of individuals with NF1 Juliana Souza, MD, Fluminense Federal University, Brazil	
16:35 - 17:00	Lessons learnt from breast cancer screening in high risk women--BRCA screening in women with NF1, when and how Eva Trevisson, University of Padova, Italy	
17:00 - 17:25	Oral manifestations in NF1 Karin Cunha Karin SG Cunha, Federal University of Minas Gerais, Brazil	
17:25 - 17:50	Beyond esthetic surgery: Electrodesiccation for cutaneous neurofibromas Hubert Weinberg, Mt. Sinai Hospital, US	
	Research	

17:50 – 18:15 Swine models for neurofibromatosis
David Largaespada, PhD, University of Minnesota, US

16:00 - 18:00 **ANIMAL MODELS (Part 2)**
Pathology of genetically engineered NF animal models: consensus report

(ROOM 201)

Co-chairs:

- Marco Giovannini, MD, PhD
- Nancy Ratner, PhD,
- Anat-Stemmer Rachamimov, MD

13:30 - 18:00 **POUR LES PATIENTS ET LEURS FAMILLES:**
Etat actuel des soins cliniques et de la recherche
Session francophone / This session will be in French only

(PETIT AMPHI)

13 :30 – 14:00 Les associations de malades et de parents contre les neurofibromatoses
(*Non for profit organizations and neurofibromatoses.*)
Jean-Michel Dubois, Annette Bakker

14:00 – 14:30 Neurofibromatose 2
(*Neurofibromatosis 2*)
Gareth Evans, Michel Kalamarides

14:30 – 15:00 Les adolescents atteints de neurofibromatose 1 et 2
(*Teen agers with neurofibromatoses 1 and 2*)
Ros Ferner, Marie Laure Armand

15:00 – 15:30 La prise en charge clinique de la neurofibromatose 1
(*Clinical management of neurofibromatosis 1*)
Eric Legius, Patrick Combemale

15:30 – 16:00 BREAK

16:00 – 16:30 Futur thérapeutique dans le domaine des neurofibromatoses
(*Therapeutic future of neurofibromatosis 1*)
Susan Huson, Pierre Wolkenstein

16:30 – 17:00 Modèles Animaux au cours des neurofibromatoses
(*Animal models in neurofibromatoses*)
Marco Giovannini, Piotr Topilko

17:00 – 17:30 Génétique des neurofibromatoses
(*Genetics in neurofibromatoses*)
Ludwine Messiaen, Dominique Vidaud

13:30 - 18:00 **CURRENT STATE OF CLINICAL CARE AND RESEARCH**
English version

(ROOM 69)

13:30 – 14:00 Neurofibromatosis Type 2
Gareth Evans, Michel Kalamarides

14:00 – 14:30 Teen agers with neurofibromatoses 1 and 2
Ros Ferner, Marie Laure Armand

- 14:30 – 15:00 Clinical management of neurofibromatosis 1
Eric Legius, Patrick Combemale
- 15:00 – 15:30 Therapeutic future of neurofibromatosis 1
Susan Huson, Pierre Wolkenstein
- 15:30 – 16:00 BREAK
- 16:00 – 16:30 Animal models in the neurofibromatoses
Marco Giovannini, Piotr Topilko
- 16:30 – 17:00 Genetics in neurofibromatoses
Ludwine Messiaen, Dominique Vidaud.
- 17 :00 – 17 :30 : Non rprofit organizations and neurofibromatoses
Jean-Michel Dubois, Annette Bakker

SATURDAY - 3 NOVEMBER 2018

08:30– 08:40	Welcome remarks	(GRAND AMPHI)
08:40 – 09:00	Platform talk: SPRINT: Phase II study of the MEK 1/2 inhibitor selumetinib (AZD6244, ARRY-142886) in children with neurofibromatosis type 1 (NF1) and inoperable plexiform neurofibromas (PN)”) Andrea Gross, MD, National Institutes of Health, National Cancer Institute, US	(GRAND AMPHI)
09:00 - 10:00	KEYNOTE LECTURE: DANNY REINBERG, PHD Howard Hughes Instit., Terry and Mel Karmazin Prof. of Biochemistry and Molecular Pharmacology, NYU Langone School of Medicine (USA) Epigenetics: One genome, multiple phenotypes	(GRAND AMPHI)
09:00 – 12:00	EUROPEAN ASSOCIATION MEETING	(PETIT AMPHI)
10:00 - 10:30	Break	
10:30 – 12:00	EUROPEAN ASSOCIATION MEETING	(PETIT AMPHI)
10:30 – 12:00	PLENARY SESSION: EPIGENETICS Co-chairs: <ul style="list-style-type: none">• Eric Pasmant, PhD, Universite Paris-Descartes, Hopital Cochin, France• Thomas De Raedt, PhD, Children’s Hospital of Philadelphia, US• Karlyne Reilly, PhD, National Cancer Institute, US Invited Speakers:	(GRAND AMPHI)
10:30 – 10:55	Thomas De Raedt, PhD Children’s Hospital of Philadelphia, US Exploiting epigenetic vulnerabilities to improve immunotherapy	
10:55 – 11:20	Josh Waterfall, PhD Curie Institute, France Learning from rare tumors: tumor immunogenicity beyond somatic mutation burden	
11:20 – 11:40	Platform talk: The genomic landscape of schwannomatosis schwannomas Sheila Mansouri, Canada	
11:40 – 12:00	Platform talk: Cutaneous and plexiform neurofibromas maintain distinct epigenetic profiles with predicted impact on tumor biology Matthew Steensma, US	

12:00 - 13:00 **Lunch break**

- 13:00 - 14:00** KEYNOTE LECTURE **(GRAND AMPHI)**
KRISTJAN JESSEN, PhD
 Professor of Developmental Neurobiology, University College, London (UK)
 Topic: Schwann Cell Biology
- 14:00 - 15:30** **PLENARY SESSION: SCHWANN CELLS AND NEUROFIBROMAS** **(GRAND AMPHI)**
- Co-chairs:
- Lu Le, MD, PhD, University of Texas, Southwestern, US
 - Juha Peltonen, MD, PhD, University of Turku, Finland
 - Eduard Serra, PhD, ICTP, Spain
- Invited Speakers :
- 14 :00 – 14 :25** Lu Le, MD, PhD
 University of Texas Southwestern Medical Center
 Developmental Origin and Spatiotemporal *NF1* Loss of Heterozygosity Leads to Different Types of Cutaneous Neurofibroma
- 14:25 – 14:50** Piotr Topilko, PhD – INSERM, France
 Pathogenic mechanisms of cutaneous neurofibromas revealed by mice carrying *NF1* loss in *Prss56*-expressing boundary cap cells
- 14 :50 – 15:10** **Platform talk:**
 Tonic ATP-mediated growth suppression in peripheral nerve glia requires arrestin-PP2 and is evaded in *NF1*
 Robert Coover, US
- 15 :10 – 15 :30** **Platform talk:**
 Trametinib in pediatric patients with neurofibromatosis type 1-associated plexiform neurofibroma: a phase I/IIa study
 Christopher L Moertel, US
- 14:00 – 16:00** **PARALLEL SESSION: SURGERY IN NF1** **(ROOM 201)**
- Co-chairs and speakers:
- Laurent Lantieri, MD, PhD l'Hôpital Européen Georges-Pompidou, FR
 - Xiaojie Hu, MD, PhD Shanghai Jiaotong University School of Medicine, CHINA
 - Reinhard Friedrich, MD, University Medical Center, Hamburg, Germany
 - Joseph Gruss, MD, Seattle Children's Hospital
- Topic 1: NF plexiform: Coping with the limits of surgical resection: short term: bleeding, nervous morbidity. Long term: inefficiency.
 Topic 2: Peripheral nerve surgery: decompression, resection, targeted indications
 Topic 3: Collaborations with other specialties and interdisciplinary approach
 Topic 4: Indications (age, locations) limitations of pediatric surgical management, long-term outcomes
 Topic 5: Resection planning: the means "modern " Planning: 3d modeling, augmented reality etc
 Topic 6: The limits of conventional plastic surgery: Indications and results VCA
- 14:00 - 15:30** **PARALLEL SESSION: PSYCHOLOGY WORKSHOP** **(ROOM 33)**
The participation to this session is by invitation
PROGRAM to be announced
- Co-Chairs :
- Stacy Martin
 - Marie-Laure Armand

15:30 - 16:00 Break

16:00 - 17:50 **PLENARY SESSION: PSYCHOSOCIAL IMPACT OF NEUROFIBROMATOSIS**

(GRAND AMPHI)

Co-chairs:

- Marie-Laure Armand, PhD, Hopital Henri-Mondor, FR
- Staci Martin, PhD, National Institutes of Health, US
- Hilda Crawford, PhD, University of Sydney, Australia

Invited Speakers:

16:00 – 16:25 Belinda Barton, PhD
University of Sydney, Australia
Supporting the psychosocial needs of individuals with neurofibromatosis: International Perspectives

16:25 – 16:50 Staci Martin, PhD
National Institutes of Health, US
Acceptance and commitment therapy (ACT) for individuals with NF1, plexiform neurofibroma tumors (PNs), and chronic pain: results from a randomized controlled trial

16:50 – 17:10 **Platform talk:**
improving quality of life (QoL) in internationally diverse patients with NF via secure live video: Results of 3 randomized controlled trials (RCTs) in adults, adolescents and patients who are deaf
Ana-Maria Vranceanu, US

17:10 – 17:30 **Platform talk :**
Prospective Patient-Reported Outcomes (PROs) Document Clinical Benefit in Children with Neurofibromatosis Type 1 (NF1) and Inoperable Plexiform Neurofibromas (PNs) on SPRINT: a Phase II Trial of the MEK 1/2 Inhibitor Selumetinib (AZD6244,ARRY-142886)
Pamela L Wolters, US

17 :30 – 17:50 NF Clinical Trials Consortium Update
Michael Fisher, MD
Children’s Hospital, Philadelphia, US

17:50 – 18:00 **POSTER ADVERTISEMENTS**

(GRAND AMPHI)

18:00 – 19:30 **BASIC SCIENCE POSTER SESSION**

(POSTER AREA – ROOM 101)

SUNDAY - 4 NOVEMBER 2018

09:00 - 10:00	KEYNOTE LECTURE PAOLO G. CASALI, MD Dir. Medical Oncology, Unit 2, Fondazione IRCCS Istituto Nazionale Tumori, Assoc. Prof. Medical Oncology University of Milan (Italy) Malignant Nerve Sheath Tumor: Improving the Prognosis	(GRAND AMPHI)
10:00 - 10:50	PLENARY SESSION: MUTATIONS IN OTHER CANCERS (PART 1) Co-chairs: <ul style="list-style-type: none">• Meena Upadhyaya, PhD, University of Cardiff, Wales, UK• Ophelia Maertens, PhD, Brigham and Women's Hospital/Harvard University, US Invited Speakers:	(GRAND AMPHI)
10:00 – 10:25	Ophelia Maertens, PhD Brigham and Women's Hospital/Harvard University, US The expanding role of NF1 in sporadic cancer and resistance to targeted therapies	
10:25 – 10:50:	Antonio Iavarone, MD Columbia University, US The landscape of molecular alterations and mechanisms of progression in NF1 gliomas	
10:50 - 11:20	Break	
11:20 - 12:00	PLENARY SESSION: MUTATIONS IN OTHER CANCERS (PART 2) Co-chairs: <ul style="list-style-type: none">• Meena Upadhyaya, PhD	(GRAND AMPHI)
11:20 – 11:40	Platform talk: PEDF dysregulation promotes proliferation and invasion in malignant melanoma with NF1 driver mutation Girish Patel, UK	
11:40 – 12:00	Platform talk: Molecular and functional consequences of somatic NF1 mutations in non-small cell lung cancers Eric Pasmant, France	
12:00 – 13:00	Lunch break	
13:00 - 14:30	NF2 AND SCHWANNOMATOSIS STATE OF THE ART (Part 1) Clinical Co-chairs: <ul style="list-style-type: none">• Michel Kalamirides, MD, PhD• Marco Giovannini, MD, PhD, UCLA, US	(PETIT AMPHI)
13:00 – 13:05	Living with NF2 – A Patient's Perspective Mckinnon Galloway, US	
13:05 – 13:30	NF2/SWN Diagnostic criteria revision Gareth Evans, MD, University of Manchester, UK	

13:30 – 14:00 Genetics of NF2/ SCHWANNOMATOSIS
Gareth Evans, MD, University of Manchester, UK

14:00 – 14:30 Current and emerging drug treatment options
Scott Plotkin, MD, PhD, Massachusetts General Hospital/Harvard University, US

13:00 - 14:30 PARALLEL SESSION: GLIOMA – IN/OUT OF NF

(GRAND AMPHI)

Co-chairs:

- Michael Fisher, MD, Children’s Hospital of Philadelphia, US
- David Walker, MD, Children’s Brain Tumour Center, UK
- Antonio Iavarone, MD, Columbia University, US

13:00 – 13:25 David Gutmann, MD, PhD
Washington University, US
New Insights into the molecular and cellular pathogenesis of optic glioma

13:25 – 13:45 **Platform talk:**
Synodos for NF1 Glioma: In-depth genomic characterization of pediatric NF1-associated gliomas reveals recurrent cooperating mutations and an aggressive tumor subset
David T. W. Jones, Germany

13:45 – 14:05 **Platform talk:**
Larger Tumor Volume is Associated with Visual Acuity Loss and Axonal Degeneration in Children with Optic Pathway Gliomas Secondary to NF1.
Robert Avery, United States

14:05 – 14:30 Amedeo Azizi, MD, PhD
University of Vienna, Austria
NF1 Optic Pathway Glioma: Treat or observe? A proposal for a randomized selection of treatment versus observation

13:00 - 14:35 PARALLEL SESSION: FUNCTIONAL GENETICS AND INTERPRETATION /CLASSIFICATION OF VARIANTS

(ROOM 201)

Co-chairs:

- Akihiko Yoshimura, PhD, Keio University, Japan
- Eric Legius, MD, PhD, University of Leuven, Belgium
- Frans Verheijen, PhD, Erasmus MC, Netherlands

13:00 – 13:25 **Katharina Wimmer, PHD**
University of Innsbruck, Austria
Splice effect assessment: the important first step of *NF1* variant interpretation

13:25 – 13:50 **Rick Van Minkelen, PhD**
Erasmus University Medical Center, Netherlands
Functional assessment of NF1 missense variants identified in individuals suspected of neurofibromatosis type 1

13:50 – 14:15 **Akihiro Yoshimura, PhD**
Keijo University, Japan
Effects of mutations in SPRED1 on Legius Syndrome and relationship to NF1

14:15 - 14:35 **Platform talk:**
Neurofibromin a new SUMO target involved in neurofibromatosis type 1 disease
Mohammed Bergoug, France

15:00 - 17:00 **NF2 AND SCHWANNOMATOSIS STATE OF THE ART (Part 2)**

(PETIT AMPHI)

15:00 – 15:20 Treatment of Vestibular Schwannomas
Michael Link, MD, Mayo Clinic, US

15:20 – 15:40 Treatment of Meningiomas
John Golfinos, MD, New York University, Langone Medical Center, US

15:40 – 16:00 Hearing Rehabilitation
Olivier Sterkers, MD, PhD, Hopital Pitie Salpetriere, FR

16:00 – 16:20 Treatment of Spinal Tumors
Andrew King, MD, University of Manchester, UK

16:20 – 16:40 Peripheral nerve schwannomas
Allan Belzberg, MD, Johns Hopkins University, US

16:40 – 17:00 Management of NF2 in China
H. Wu, MD, PhD, 9th People's Hospital Shanghai Jiao Tong University School of Medicine, China

15:00 - 17:15 **PARALLEL SESSION: LEARNING DISABILITIES**

(GRAND AMPHI)

Co-chairs:

- Thijs van der Vaart, MD, PhD, Erasmus University Medical Center, Netherlands
- Jonathan Payne, PsyD, Murdoch Children's Hospital, Australia
- Nicole Ullrich, MD, PhD, Boston Children's Hospital, Harvard University, US

Invited speakers:

15:00 – 15:25 Shruti Garg, PhD
University of Manchester, UK
Multi parametric imaging and behavior in an early-phase mechanism trial of simvastatin for NF1-autism

15:25 – 15:50 Stavros Stivaros, PhD
University of Manchester, UK
Multi parametric imaging and behavior in an early-phase mechanism trial of simvastatin for NF1-autism

15:50 – 16:15 Hilde Brems, PhD
University of Leuven, Belgium
Animal model data on *SPRED1*

16:15 – 16:35 **Platform talk:**
Genotype and behavioral phenotypes in children and adolescents with Neurofibromatosis type 1
Andre Rietman, The Netherlands

16:35 – 16:55 **Platform talk:**
Risk of Psychosocial, Cognitive and Health Impairments in Adult Survivors of Childhood Glioma with Neurofibromatosis Type 1
Peter de Blank, United States

16:55 – 17:15 **Platform talk:**
Understanding the NF1 experience and the priorities of the NF1-affected individuals and families for cognitive and social-emotional research
Tess Kennedy, United States

15:00 - 17:15 PARALLEL SESSION: GENOTYPE/PHENOTYPE OF NF1

(ROOM 201)

Co-chairs:

- Ludwine Messiaen, PhD, University of Alabama at Birmingham, US
- Hildegard Kehrer-Sawatzki, PhD University of Ulm, Germany
- Douglas Stewart, MD, National Cancer Institute, US

Invited Speakers:

- 15:00 – 15:25 Ludwine Messiaen, PhD
University of Alabama at Birmingham, Us
Genotype-phenotype correlations in NF1: the current state of knowledge
- 15:25 – 15:50 Ype Elgersma, PhD
Erasmus University Medical Center – Netherlands
Identification of the factors underlying the cognitive variance associated with NF1
- 15:50 – 16:15 Jung Kim, PhD, National Cancer Institute, US
Looking beyond the lamppost: exome sequencing in 92,296 individuals in a single US healthcare system to investigate prevalence, penetrance, and phenotype in NF1 and Legius syndrome
- 16:15 – 16:35 **Platform talk:**
Comprehensive screening of NF1 missense mutations
James Walker, United States
- 16:35 – 16:55 **Platform talk:**
NF1 deficiency is linked to estrogen signaling in breast cancer
Matthew Steensma, United States
- 16:55 – 17:15 **Platform talk:**
Genetic basis of variable clinical expression in NF1
Audrey Sabbagh, France

17:15 - 17:25 POSTER ADVERTISEMENTS

(GRAND AMPHI)

17:25 – 18:55 CLINICAL POSTER SESSION

(POSTER AREA – ROOM 8)

MONDAY - 5 NOVEMBER 2018

- 08:30 - 09:30** KEYNOTE LECTURE: **(GRAND AMPHI)**
PIER PAOLO PANDOLFI, MD, PHD
Director, Cancer Center and Cancer Research Institute – Beth Israel Deaconess Medical Center, Chief, Division of Genetics, Dept. Of Medicine, BIDMC (USA)
Topic: Translational mouse: from genetic models to the mouse hospital, and co-clinical trials for understanding and treatment of cancer
- 09:30 – 11:00** **PLENARY SESSION: MICROENVIRONMENT** **(GRAND AMPHI)**
Co-chairs:
 - Piotr Topilko, PhD , INSERM, France
 - D. Wade Clapp, MD, Indiana University, US
 - Alison Lloyd, PhD, University College, London, UK
- 9:30 – 9:55** Juha Peltonen, MD, PhD, University of Turku, Finland,
The cellular and extracellular microenvironment of cutaneous neurofibromas
- 9:55 – 10:20:** Nancy Ratner, PhD, Cincinnati Children’s Hospital
Cxcr3 expressing leukocytes are necessary for neurofibroma formation in mice
- 10:20 – 10:40** **Platform talk:**
Dermal fibroblast-derived exosomes enhance angiogenesis in a tissue-engineered skin model of neurofibromatosis type 1
Vincent Roy, Canada
- 10:40 – 11:00:** **Platform talk:**
Targeting the Hyaluronan-Rich Peripheral Nerve Sheath Tumor Microenvironment to Improve Drug Efficacy and Delivery
Bryant John Keller
- 09:30 - 10:50** **PARALLEL SESSION: NF2/SCHWANNOMATOSIS** **(PETIT AMPHI)**
Co-chairs :
 - Michel Kalamarides, MD, PhD
 - Marco Giovannini, MD, PhD
- 09:30 – 09:40** **Platform talk:**
A Phase 0 Pharmacodynamic and Pharmacokinetic Study of Everolimus in Vestibular Schwannoma (VS) and Meningioma Patients
Matthias A. Karajannis, US
- 9 :40 – 9 :50** **Platform talk:**
A single arm phase 2 study of the dual mTORC1/mTORC2 inhibitor vistusertib provided on an intermittent schedule for neurofibromatosis 2 patients with progressive or symptomatic meningiomas
Scott Plotkin, US
- 9 :50 – 10 :00** **Platform talk:**
Management of NF2-associated vestibular schwannomas in children and young adults: influence of surgery on tumor volume and growth rate
Isabel Gugel, Germany
- 10:00 – 10:50** Clinical Cases Panel Discussion

- Thomas Roland,, MD, New York University, US
- Michael Link, MD, Mayo Clinic, US
- Brad Welling, MD, PhD, Massachusetts Eye and Ear Institute, Harvard University, US
- Scott Plotkin,, MD, PhD, Massachusetts General Hospital, US
- John Golfinos,, MD, New York University, US
- Gareth Evans,, MD, Univ. of Manchester, UK

10:50 – 11:20 Break

11:20 - 12:50 PLENARY SESSION: PAIN AND PRURITIS IN NEUROFIBROMATOSIS

(GRAND AMPHI)

Co-chairs:

- Allan Belzberg, MD, Johns Hopkins University, US
- Rosalie Ferner, MD , SGuy's and St. Thomas' NHS Foundation Trust and King's College, UK
- Scott Plotkin, MD, PhD, Massachusetts General Hospital, US

Invited Speakers:

- 11:20 – 11:45 Laurent Misery, MD – Universite de Bretagne Occidentale, France
Pathophysiology of Itch
- 11:45 – 12:05 Nadine Attal, MD, PhD – Universite Versailles Saint-Quentin, France – 12:00
Therapeutic management of neuropathic pain
- 12:05 – 12:30 Michael Caterina, MD, PhD – Johns Hopkins Medical Center, US
Biology of neuropathic pain and potential links to NF
- 12:30 – 12:50 **Platform talk:**
Molecular mechanisms underlying schwannomatosis pain
Larry Sherman, US

12:50 – 13:30 Lunch break

13:30 - 14:30 KEYNOTE LECTURE:

(GRAND AMPHI)

CAROLINE ROBERT, MD, PHD

Head of Dermatology Unit, Institute Gustave-Roussy, Villejuif, Board Member, European Association of Onco-Dermatology; Melanoma Board Secretary for European Organization for the Research and Treatment of Cancer; French Society of Dermatology and Venereology, AACR, ASCO (France)

Topic: Targeted Therapy in Melanoma

14:30 - 16:30 PLATFORM PRESENTATIONS - BASIC

(GRAND AMPHI)

Chairs:

- Yuan Zhu, PhD, Children's National Medical Center, US
- Conxi Lazaro, PhD, Institut Catala d'Oncologia , Spain

14:30 – 14:50 Platform talk:

Transposon Mutagenesis and CRISPR/Cas9 Screening Reveal Pathways Driving Malignant Peripheral Nerve Sheath Tumor Development and Maintenance
German Luis Velez Reyes, US

- 14:50 – 15:10 **Platform talk:**
Early development and autism risk in Neurofibromatosis Type 1
Anna Kolesnik, UK
- 15:10 – 15:30 **Platform talk:**
Antisense directed gene therapy to treat intragenic Neurofibromatosis Type I (NF1) mutations
Michael Daniel, US
- 15:30 – 15 :50 **Platform talk:**
Feasibility of Gene Replacement Therapy in NF1-related MPNST
Verena Staedtke, US
- 15 :50 – 16 :10 **Platform talk:**
Preclinical Assessment of MEK1/2 Inhibitors for Neurofibromatosis Type 2-Associated Schwannomas Reveals Differences in Efficacy and Drug Resistance Development
Cristina Fernandez-Valle, US
- 16:10 – 16:30 **Platform talk:**
Novel drug discovery for NF2-deficient meningiomas: Brigatinib causes tumor shrinkage in NF2-deficient meningiomas
Long-Sheng Chang, US

14:30 - 16:30 PLATFORM PRESENTATIONS – CLINICAL

(PETIT AMPHI)

Co-chairs:

- David Viskochil, MD, PhD, University of Utah, US
- Patrick Combemale, MD, Cancer Research Center of Lyon, France

- 14:30 – 14:50 **Platform talk:**
NF105: A Neurofibromatosis Clinical Trials Consortium (NFCTC) phase II study of Cabozantinib (XL184) for neurofibromatosis type 1 (NF1) associated plexiform neurofibromas
Michael Fisher, US
- 14:50 – 15:10 **Platform talk:**
Clinical presentation and 5 year survival of 83 individuals with NF1 associated malignant peripheral nerve sheath tumour in a national Neurofibromatosis Centre
Rosalie Ferner, UK
- 15:10 – 15:30 **Platform talk:**
Risk of Subsequent Neoplasms (SNs) after a primary tumor in patients with NF1
Michael Fisher, US
- 15:30 – 15:50 **Platform talk:**
Dorsal root ganglia volume differentiates schwannomatosis and neurofibromatosis type 2
Tim Godel, Germany
- 15:50 – 16:10 **Platform talk:**
Use of an NF2 genetic severity score to incorporate genotype into routine patient care
Dorothy Halliday, UK
- 16:10 – 16:30 **Platform talk:**
Smile reanimation using the lengthening temporalis myoplasty in patients with facial paralysis
Andre Panossian, US

16:45 - 18:15 **PLENARY SESSION: MEDICAL AND SURGICAL CARE:
A MULTIDISCIPLINARY APPROACH FOR NF**

(GRAND AMPHI)

Co-chairs:

- Michel Kalamarides, MD, PhD, Hopital Pitie Salpetriere, FR
- Bruce Korf, MD, PhD, University of Alabama at Birmingham, US
- Jaishri Blakeley, MD, Johns Hopkins University, US

16:45 - 18:40 **PARALLEL SESSION: BIOLOGY OF NF2 AND SCHWANNOMATOSIS**

(PETIT AMPHI)

Co-chairs:

- Helen Morrison, PhD, Leibniz Institute on Aging, Germany
- Andrea McClatchey, PhD, Harvard University US
- Jeremie Vitte, PhD, UCLA, US

Invited Speakers:

- 16:45 – 17:10 Jeremie Vitte, PhD – UCLA, US
Late inactivation of Smarcb1 and additional Nf2 inactivation are necessary for schwannoma development in schwannomatosis patients
- 17:10 – 17:35 Tina IZARD, PhD – Scripps Research Institute, US
Dissecting the conformational changes in merlin
- 17:35 – 18:00 Alison Lloyd, PhD – University College, London, UK
Peripheral nerve microenvironment/schwann cells
- 18:00 – 18:20 **Platform talk:**
LZTR1 is a MAM protein regulating mitochondrial calcium and respiration in Schwann cells
Asha Kiran Akula, Germany
- 18:20 – 18:40 **Platform talk:**
Merlin/ERM proteins regulate growth factor-induced macropinocytosis and receptor recycling by organizing the plasma membrane:cytoskeleton interface
Christine Chiasson-Mackenzie, US

TUESDAY - 6 NOVEMBER 2018

08:30 - 09:30	ORAL POSTER PRESENTATIONS	(GRAND AMPHI)
09:30 – 10 :00	KEYNOTE LECTURE: YVES LEVY, MD, PHD Chairman, CEO Institute National de la Sante et de la Recherche Medicale (INSERM) (France) Topic: Plan France Médecine Génomique 2025	(GRAND AMPHI)
10:00 – 11:10	PLENARY SESSION: NEUROFIBROMATOSIS: PAST, PRESENT AND FUTURE Co-Chairs: <ul style="list-style-type: none">• Annette Bakker, PhD, Children’s Tumor Foundation, US• Maurizio Clementi, MD, University of Padova, IT Invited Speakers:	(GRAND AMPHI)
10:00 – 10:20	Vincent Riccardi, MD – Neurofibrotosis Institute, US	
10:20 – 11:10	Gareth Evans, MD – St. Mary’s Hospital, University of Manchester, UK)	
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11:10 – 11:40	Break	
11:40 - 12:25	PLENARY SESSION: NEUROFIBROMATOSIS: PRESENT, PAST AND FUTURE	(GRAND AMPHI)
11:40 – 12:05	Susan Huson, MD – Consultant Geneticist , UK	
12:05 – 12:25	Luis Parada, PhD – Memorial Sloan Kettering Cancer Center, US	
12:25 - 13:45	PLENARY SESSION: REVISION OF DIAGNOSTIC CRITERIA	(GRAND AMPHI)
	Chairs: <ul style="list-style-type: none">• Eric Legius, MD, PhD – University of Leuven, Belgium• Scott Plotkin, MD, PhD – Massachusetts General Hospital/Harvard University, US• Gareth Evans, MD – St. Mary’s Hospital/University of Manchester, UK• Susan Huson, MD – Consultant Geneticist, UK	
13:45 - 14:00	Congress Adjourned	
15:00 - 18:00	REiNS session (By invitation) Response Evaluation in Neurofibromatosis and Schwannomatosis	(ROOM 101)