Monday 25 January 2021 (time zone CET)

16.00-16.05 **Opening ANR2021**
Frank Speleman and Rogier Versteeg

16.05-17.05 **Major Symposium 1: Progress in treatment and challenges for preclinical studies**
Moderator: Angelika Eggert, Berlin, Germany and & Godfrey Chan, China

This session will provide an update on the major treatment studies for high risk neuroblastoma and is intended for both clinical and basic researchers. This session should define the major questions in treatment of neuroblastoma, both from a basic and clinical point of view. Relapse treatment studies will be reviewed, as well as local tumor control by surgery and radiotherapy and the role of new imaging technologies.

16.05-16.15 Introduction to symposium
Angelika Eggert, Berlin, Germany

16.15-16.25 Introduction of new HR-NBL2-SIOPEN trial
Dominique Valteau-Couanet, Villejuif, France

16.25-16.35 Introduction to Current and Future COG High Risk Trials
Rochelle Bagatell, Philadelphia, US

16.35-16.45 Current international relapse concepts/early clinical trials
Lucas Moreno, Barcelona, Spain

16.45-16.55 Low and intermediate risk neuroblastoma
Barbara Hero, Koln, Germany

16.55-17.05 MS1.1 Surviving High-Risk Neuroblastoma: A Preliminary Descriptive Report from Project LEAHRN (Late Effects After High-Risk Neuroblastoma)
Lisa Diller, Boston, USA

17.05 - 17.15 **Break**

17.15- 18.35 **Major Symposium 2: Neuroblastoma as a developmental disorder**
Moderator: Olivier Delattre, Paris, France and & Herman Rohrer, Germany

Neuroblastoma is marked by inter-tumor heterogeneity defining stages with highly divergent outcomes, but also by intra-tumour heterogeneity which may underlie relapse development. New technologies currently redefine the normal development of the sympatho-adrenergic lineage. This session will integrate the latest insights in normal development, tumor heterogeneity and clinical behaviour.

17.15-17.20 Introduction to symposium
Olivier Delattre, Paris, France

17.20-17.35 Introduction on normal sympatho-adrenergic lineage development
Igor Adameyko, Stockholm, Sweden

17.35-17.50 MS2.1 Developmental and oncogenic programs in neuroblastomas dissected by single-cell analysis
Selina Jansky, Heidelberg, Germany

17.50-18.05 MS2.2 Single-cell RNA sequencing of human neuroblastoma reveals Schwann cell precursors as putative cancer stem cells
Thale Kristin Olsen, Stockholm, Sweden

18.05-18.20 MS2.3 Dissecting neuroblastoma tumor heterogeneity and cell plasticity by single cell RNAseq
Cecile Thirant, Paris, France

18.20-18.35 MS2.4 Drug resistance and phenotype of MES- and ADRN-type neuroblastoma cells faithfully reflect consecutive stages of normal adrenergic lineage development
Johan Van Nes, Amsterdam, the Netherlands

18.35-18.45 Break

18.45 – 19.45 Parallel sessions

PA1: Treatment relapse Neuroblastoma

18.45–19.00 PA1.1 Relapsed/refractory pediatric neuroblastoma: excellent tolerability and sustained responses with temozolomide-based regimens
Lucy Métayer, Villejuif, France

19:00-19.15 PA1.2 Risk classification for patients with first recurrence of stage 4 neuroblastoma
Angela Ernst, Cologne, Germany

19.15-19.30 PA1.3 Randomized Phase II Trial of MIBG vs. MIBG/Vincristine/Irinotecan vs. MIBG/Vorinostat for Patients with Relapsed/Refractory Neuroblastoma:
A Report from the New Approaches to Neuroblastoma Therapy
Steven DuBois, Boston, USA

19.30-19.45 PA1.4 Long Term Survival for Patients with Central Nervous System Metastases Following Treatment with Intraventricular Radiolabeled Omburtamab: Results of Trial 03-133
Kim Kramer, New York, USA

PA2: Omics, new concepts

18.45-19.00 PA2.1 Contribution of TWIST1 in the aggressiveness of neuroblastoma by modulation of the tumor-stroma crosstalk
Maria-Vittoria Sepporta, Lausanne, Switzerland

19.00-19.15 PA2.2 Targeting Fatty Acid Transport in MYCN-amplified Neuroblastoma
Ling Tao, Houston, USA

19.15-19.30 PA2.3 MYCN regulates metabolism through vesicular transfer of glycolytic kinases
Alexia Tsakaneli, London, UK

19.30-19.45 PA2.4 Cellular and gene signatures of tumor-infiltrating dendritic cells and natural killer cells predict favorable clinical outcome of neuroblastoma
Ombretta Melaiu, Rome, Italy

PA3: Neuroblastoma as development disorder

18.45-19.00 PA3.1 Single cell transcriptome analysis decodes development trajectory of neural crest cells
Ran Yang, Shanghai, China

19.00 -19.15 PA3.2 A single cell atlas of the developing murine adrenal gland
Evelyn Hanemaaijer, Utrecht, The Netherlands

19.15-19.30 PA3.3 ASCL1 activation by dephosphorylation can direct a genome-wide re-engagement of a latent differentiation programme in neuroblastoma
Lydia Parkinson, Cambridge, UK

19.30-19.45 PA3.4 The development of a human embryonic stem cell derived differentiation model to study normal and neuroblastoma development
Stéphane Van Haver, Ghent, Belgium

PA4: Clinical – Neuroblastoma frontline therapy

18:45-19.00 PA4.1 Natural history of ganglioneuroma (GN) and intermixed ganglioneuroblastoma (iGNB): An International Neuroblastoma Risk Group (INRG) project
Paola Angelini, Sutton, UK

19.00-19.15 PA4.2 Long-term health status of high-risk neuroblastoma survivors treated with high-dose chemotherapy and hematopoietic stem cell transplantation
Sandrine Haghiri, Villejuif, France

19.15-19.30 PA4.3 Neuroblastoma Patient-Derived Cell Lines and Xenografts in the COG/ALSF Childhood Cancer Repository
Kristyn McCoy, Lubbock, USA

19.30-19.45 PA4.4 The incidence of neuroblastoma cases before and after screening in Japan
Tomoko Iehara, Kyoto, Japan

Tuesday 26 January 2021 (time zone CET)

15.55-16.00 **Presidential address**
Gudrun Schleiermacher, Paris, France

16.05-17.10 **Major Symposium 3: A deep look into neuroblastoma**
Moderators: Gudrun Schleiermacher, Paris, France and Susanne Schlisio, Solna, Sweden

Single cell analysis is revolutionizing cancer research. The results for neuroblastoma will be presented in this session, offering insight in intra-tumor heterogeneity, immune and stromal infiltrate and clinical consequences.

16.05-16.10 and introduction to symposium
Gudrun Schleiermacher, Paris, France

16.10-16.25 MS3.1 Clonal heterogeneity before treatment underlies spatial and temporal evolution in neuroblastoma
Gunes Gundem, New York, USA

16.25-16.40 MS3.2 Molecular diagnostics and targeted therapy of neuroblastoma in light of intratumour heterogeneity
Karin Schmelz, Berlin, Germany

16.40-16.55 MS3.3 Single cell DNA sequencing and sequential circulating tumor DNA analysis highlight intratumor genetic heterogeneity and clonal evolution under targeted therapies in neuroblastoma
Angela Bellini, Paris, France

16.55-17.10 MS3.4 Single-cell RNA-sequencing of peripheral neuroblastic tumors identifies an aggressive transitional cell state at the junction of an adrenergic-mesenchymal transdifferentiation trajectory
Daniel Carter, Randwick, Australia

17.10-17.15 Break

17.15-18.20 **Major Symposium 4: Neuroblastoma tumour biology and genomics: from bench to the clinic**
Moderators: John Maris, Philadelphia, USA and Matthias Fischer, Cologne, Germany

This session will focus on insights from novel layers of omics information of gene mutations, structural defects and other high throughput data. It will address the relation between these defects and relapse potential and clinical development, as well as functional studies of these defects. Potential drug targets and new bioinformatic tools to study them will be addressed.

17.15-17.20 Introduction to symposium
John Maris, Philadelphia, USA and Matthias Fischer, Cologne, Germany

17.20-17.35 MS4.1 The BRIP1 17q dependency gene in neuroblastoma: from fork stability to translation
Suzanne Vanhauwaert, Ghent, Belgium

17.35-17.50 MS4.2 A multi-omic surfaceome study identifies DLK1 as an epigenetically regulated protein and immunotherapeutic target in neuroblastoma
Amber Weiner, Philadelphia, USA

17.50-18.05 MS4.3 The evolution of MDM2 and MYCN amplifications in neuroblastoma
Carolina Rosswog, Cologne, Germany

18.05-18.20 MS4.4 The transcriptional co-repressor Runx1t1 is essential for N-myc-driven neuroblastoma tumorigenesis
Murray Norris, Kensington, Australia

18.20-18.30 Break

18.30-19.30 Parallel sessions

PA5: Heterogeneity in Neuroblastoma

18.30-18.45 PA5.1 Single cell RNA-sequencing analysis of tumor heterogeneity and dynamic adaptive transcriptome changes in a MYCN driven zebrafish model
Lisa Depestel, Ghent, Belgium

18.45-19.00 PA5.2 Cross-talk between Tumor Associated Macrophages (TAM) and Cancer Associated Fibroblasts (CAF) in Neuroblastoma (NB) Contributes to a Pro-tumorigenic Microenvironment (TME) that Promotes TAM
Kevin Louault, Los Angeles, USA

19.00-19.15 PA5.3 Self-renewing neuroblastoma cells of the bone marrow share a mesenchymal phenotype which is associated with poor outcome: an NCRI CCL CSG Neuroblastoma Group Study
Susan Burchill, Leeds, UK

19.15-19.30 PA5.4 The landscape and evolution of somatic mutations captured in the NEPENTHE precision medicine clinical trial for relapsed high-risk neuroblastoma
Esther Berko, Philadelphia, USA

PA6:Targeted therapy

18.30-18.45 PA6.1 APR-246, which Restores p53 Function, is Highly Active against Alternative Lengthening of Telomere (ALT) Neuroblastoma Cell Lines and PDXs
Shawn Macha, Lubbock, USA

18.45-19.00 PA6.2 Integration of High-Throughput Drug Screening on Patient-Derived Organoids into the Princess Máxima Center iTHER Precision Medicine Program: The Future is Now!
Karin Langenberg, Utrecht, the Netherlands

19.00-19.15 PA6.3 Zero Childhood Cancer (ZERO): A comprehensive genomic, high-throughput drug screening and personalised xenograft modelling platform for high-risk cancer including relapsed/refractory high-risk neuroblastoma
Alvin Kamili & Toby Trahair, Sydney, Australia

19.15-19.30 PA6.4 Synergistic antitumor effects of combining selective CDK7 and BRD4 inhibition in neuroblastoma
Malgorzata Krajewska, Boston, USA

PA7: Liquid biopsies

18.30-18:45 PA7.1 Combined blood and bone marrow cell-free DNA and disseminated tumor cell detection for sensitive response monitoring and early relapse detection in high-risk

neuroblastoma patients
Sabine Taschner-Mandl, Vienna, Austria

18.45-19.00 PA7.2 Detection of actionable genetic alterations in cell-free DNA of neuroblastoma patients enrolled in the MAPPYACTS study
Gudrun Schleiermacher, Paris, France

19.00-19.15 PA7.3 Hypermethylated RASSF1A and tumor specific DNA breakpoints as circulating tumor markers for detection of minimal residual disease
Lieke Van Zogchel, Utrecht, the Netherlands

19.15-19:30 PA7.4 Combining genomics and ultra-sensitive bone marrow assessment for risk stratification in high-risk metastatic neuroblastoma: a HR-NBL1/SIOPEN study
Stefan Fiedler, Vienna, Austria

PA8: Immune therapy

18.30-18.45 PA8.1 Leveraging an immunocompetent, MYCN-driven, non-germline GEM model for neuroblastoma and CyTOF mass cytometry to investigate immunosuppressive mechanisms and response to immunotherapy
Marie Menard, San Francisco, USA

18.45-19.00 PA8.2 Analysis of immune checkpoints in patients with high-risk neuroblastoma treated with dinutuximab beta with and without IL-2
Sascha Troschke-Meurer, Greifswald, Germany

19.00-19.15 PA8.3 The antibody-drug conjugate D3-GPC2-PBD is potently efficacious against diverse preclinical models of neuroblastoma and other cancers via engagement of a tumor-specific conformational GPC2 epitope
Kristopher Bosse, Philadelphia, USA

19.15-19:30 PA8.4 41BB or CD28 driven disialoganglioside (GD2)-specific CAR-T, but not T-cell engaging bispecific antibody, induces fatal neurotoxicity in mice
Brian Santich, New York, USA

18:30-19:30 Symposium: Stem cell mobilization and transplantation in pediatric oncology: lessons learned from clinical practice
Chairs: Kathelijne Kraal, Utrecht, the Netherlands and Max van Noesel, Utrecht, the Netherland
Sponsored by Sanofi Genzyme

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18:30-18:50 Autologous stem cell transplantation in pediatric oncology patients; poor mobilisers, plerixafor en (PEG) filgrastim
Milou Rozeman, medical student, Princess Máxima Center for pediatric oncology, Utrecht

18:50-19:10 Apheresis and autologous transplants for neuroblastoma: lessons learned
Greg Yanik (HR NBL COG), | pediatric blood and bone marrow transplantation. Michigan medicine, University of Michigan, Ann Arbor, Michigan

19:10-19:30 Coordination of apheresis by a nurse practitioner, single center experience (pegfilgrastim pilot)
Antoinette Jaspers-Bakker | nurse practitioner, Princess Máxima Center for pediatric oncology, Utrecht

Wednesday 27 January 2021 (time zone CET)

21.00-22.05 **Major Symposium 5: Targeted therapy: ALK and other targets in personalized medicine**
Moderators: Yael Mossé, Philadelphia, USA and Michelle Haber, Sydney, Australia

ALK inhibition is quintessential for the development of precision medicine in neuroblastoma: early development, unclear early clinical results followed by more effective strategies and the associated challenge of resistance. Many other targets are piloted from bench to bedside and will be discussed.

21.00-21.05 Introduction to symposium
Yael Mossé, Philadelphia, USA and Michelle Haber, Sydney, Australia

21.05-21.20 MS5.1 Phase 1 trial of Lorlatinib in patients with relapsed/refractory ALK-driven neuroblastoma
Kelly Goldsmith, Atlanta, USA

21.20-21.35 MS5.2 Arginine depletion therapy enhances efficacy of chemotherapy in delaying tumour development and increasing survival in the Th-MYCN mouse model of neuroblastoma
Ruby Pandher, Sydney, Australia

21.35-21.50 MS5.3 Targeting transcription-replication conflicts in MYCN-driven neuroblastoma
Gabriele Büchel, Wurzburg, Germany

21.50-22.05 MS5.4 Therapeutic vulnerabilities in the DNA damage response for the treatment of ATRX mutant neuroblastoma
Sally George, London, UK

22.05-22:15 Break

22.15-23.20 **Major symposium 6 (Parallel with MS7 !): Gene regulatory networks and epigenetics: from basic science towards novel therapeutic strategies**
Moderators: Kimberly Stegmaier, Boston, USA and Rani George, Boston, USA

Epigenetic networks are being clarified in neuroblastoma. They are linked to drug resistance and transcription factor networks that control neuroblastoma. This session will highlight these developments and the therapeutic potential of drugs interfering with epigenetics.

22.15-22.20 Introduction to symposium
Kimberly Stegmaier, Boston, USA and Rani George, Boston, USA

22.20-22.35 MS6.1 Extrachromosomal circular MYCN amplification: structure, regulation and genomic remodeling
Konstantin Helmsauer, Berlin, Germany

22.35-22.50 MS6.2 Three-dimensional analysis of MYCN function in neuroblastoma
Martin Eilers, Wurzburg, Germany

22.50-23.05 M6.3 Distinct roles of noradrenergic core regulatory circuitry transcription factors in neuroblastoma cell identity
Isabelle Janoueix-Lerosey, Paris, France

23.05-23.20 MS6.4 Cell lineage predicts response to therapy in neuroblastoma
Rani George, Boston, USA

22.15-23.20 Major Symposium 7 (Parallel with MS6 !): Immune therapy of neuroblastoma
Moderators: Julie Park, Seattle, USA and Holger Lode, Greifswald, Germany

This session will present successes and challenges of immune therapy in neuroblastoma including updates on anti-GD2 protocols and pilot studies. The session will include studies for other targets of antibody-mediated therapy and CART approaches that are being developed in early clinical trials

22.15-22.20 Introduction to symposium
Julie Park, Seattle, USA and Holger Lode, Greifswald, Germany

22.20-22.35 MS7.1 Academic, Phase I/II Trial on T Cells Expressing a Third-Generation GD2 Chimeric Antigen Receptor and Inducible Caspase-9 Safety Switch for Treatment of Relapsed/Refractory High-Risk Neuroblastoma
Francesca Del Bufalo, Rome, Italy

22.35-22.50 MS7.2 Pharmacodynamic effects of IL-2 in the treatment with a long-term infusion of anti-GD2 antibody dinutuximab beta: Role in outcome and toxicity in high-risk relapsed/refractory neuroblastoma patients. Results of a randomized SIOPEN-study
Holger Lode, Greifswald, Germany

22.50-23.05 MS7.3 The Composition, States and Dynamics of Microenvironmental Landscape of High-Risk Neuroblastoma Revealed by Single-Cell RNA Sequencing
Waleed Kholosy, Utrecht, the Netherlands

23.05-23.20 MS7.4 GD2 is a Macrophage Checkpoint Molecule and Combined GD2/CD47 Blockade Results in Synergistic Effects and Tumor Clearance in Xenograft Models of Neuroblastoma and Osteosarcoma
Robbie Majzner, Palo Alto, USA

23.20-23.30 Break

23.30-00:30 Parallel sessions

<u>PA9: Genomics: new genes and concepts</u>

23.30-23.45 PA9.1 The neuroblastoma dependency factor RRM2 is regulated during sympathoblast differentiation and represents a synergistic drug target for high-risk neuroblastoma
Carolina Nunes, Ghent, Belgium

23.45-00.00 PA9.2 The Yes-Associated Protein suppresses Harakiri to promote therapy resistance under tumor environmental stress in high-risk neuroblastoma
Jenny Shim, Atlanta, USA

00.00-00.15 PA9.3 Functional genomic screens identify the nuclear export factor NXT1 as a therapeutic target in MYCN-amplified neuroblastoma
Clare Malone, Boston, USA

00.15-00.30 PA9.4 Variations of DNA mismatch repair genes in hypermutated neuroblastoma
Audrey Petit, Paris, France

PA10: Targeted therapy: ALK resistance and new strategies

23.30-23.45 PA10.1 Adrenergic and Mesenchymal neuroblastoma cells have opposite resistance to ALK inhibitors and TRAIL, allowing dual therapy to impede resistance and relapse development
Ellen Westerhout, Amsterdam, the Netherlands

23.45-00.00 PA10.2 Activation of downstream signalling pathways is a mechanism of ALK inhibitor resistance in neuroblastoma
Mareike Berlak, Berlin, Germany

00.00-00.15 PA10.3 New therapeutic strategies for neuroblastoma: Targeting Gal-3BP with a highly potent Antibody-Drug Conjugate
Emily Capone, Chieti, Italy

00:15-00.30 PA10.4 The astatine-labeled PARP inhibitor [211At]MM4 induces complete and durable responses in neuroblastoma patient derived xenograft (PDX) models
Minu Samanta, Philadelphia, USA

PA11: Core Regulatory Circuitries

23.30-23.45 PA11.1 Efficacious targeting of TERT-rearranged neuroblastoma with BET bromodomain inhibitor and proteasome inhibitor combination therapy
Tao Liu, Kensington, Australia

23.45-00.00 PA11.2 SOX11 as guardian of epigenetic plasticity in neuroblastoma
Bieke Decaesteker, Ghent, Belgium

00.00-00.15 PA11.3 The neuroblastoma specific lincRNA NESPR controls noradrenergic cell identity and neuroblastoma cell survival
Eric James Bony, Ghent, Belgium

PA12: Immune landscape of neuroblastoma

23.30-23.45 PA12.1 Long-term follow-up of a Phase III Study of ch14.18 (Dinutuximab) Plus Cytokines for Patients with High-risk Neuroblastoma: Children's Oncology Group Study ANBL0032
Alice Yu, San Diego, USA

23.45-00.00 PA12.2 A Phase II Trial of Hu14.18K322A in Combination with Induction Chemotherapy in Children with Newly Diagnosed High-Risk Neuroblastoma: An Update on Early Response and EFS
Wayne Furman, Memphis, USA

00.00-00.15 PA12.3 Haploidentical stem cell transplantation and subsequent immunotherapy with anti-GD2 antibody for patients with relapsed metastatic neuroblastoma
Tim Flaadt, Tuebingen, Germay

00.15-00.30 PA12.4 Discovery and Immunotherapeutic Targeting of Lineage-restricted Major Histocompatibility Complex (MHC) Antigens in Neuroblastoma
Mark Yarmarkovich, Philadelphia, USA

PA13: Hot and cold tumours

23.30-23.45 PA13.1 High Throughput Proteomic Profiling of the Cell Surfaceome Identifies PTK7 as a Novel Immunotherapeutic Candidate for Neuroblastoma
Kelly Goldsmith, Atlanta, USA

23.45-00.00 PA13.2 Particular poor treatment outcome of high-risk patients with low affinity FCGR2A/3A and IL-2-related high regulatory T cell levels during dinutuximab beta long-term infusion in two independent cohorts
Sascha Troschke-Meurer, Greifswald, Germany

00.00-00.15 PA13.3 Immune correlative markers in refractory or relapsed neuroblastoma patients treated with irinotecan/temozolomide/dinutuximab immunotherapy
Mitchell Diccianni, San Diego, USA

00.15-00.30 PA13.4 GD2-directed bispecific trifunctional antibody demonstrates therapeutic activity in a metastasized murine neuroblastoma model
Felix Zirngibl, Berlin, Germany

23:30 – 00:30 Symposium: Addressing Misinformation in Healthcare
Sponsored by United Therapeutics

United Therapeutics CORPORATION

Misinformation in healthcare has become increasingly common as technology continues to be more ingrained in our everyday lives. We will explore misinformation in pediatric oncology and provide helpful approaches to tackle difficult family discussions related to this topic.
Andrea Ramirez, MPH, MSN, United Therapeutics

00:30 -00:45 Closing ceremony
Frank Speleman and Rogier Versteeg