

ANNUAL MEETING

NUCLEIC ACID THERAPEUTICS: GENETIC INDICATIONS AND BEYOND

TRANSLATUM, MUNICH | 6-8 MARCH 2024

PROGRAMME

WEDNESDAY 6 MARCH

12:00-13:00	Registration
13:00-13:10	Presidential Welcome Address & Welcome of Organizer
13:10-14:30	Session 1: Targeting delivery of RNA Therapeutics Chair: Ernst Wagner, LMU, Munich
	13:10-13:30 Olivia Merkel, <i>LMU, Munich</i> INV01: Artificial Intelligence for the Design of New RNA Nanocarriers
	13:30-13:50 Ulrich Lächelt, University of Vienna INV02: Xenopeptides for the delivery of CRISPR/Cas9 ribonucleoproteins
	13:50-14:10 Christian Plank, <i>Ethris, Munich</i> <u>INV03:</u> Modulation of host immunity in the airways with interferon lambda encoding mRNA
	14:10-14:20 Mina Yazdi , <i>LMU, Munich</i> <u>OR01</u> : In vivo endothelial cell gene silencing by siRNA-LNPs tuned with lipoamino bundle chemical and ligand targeting
	14:20-14:30 Olympia Bikou , <i>LMU</i> , <i>Munich</i> <u>OR02</u> : Aerosolized gene and oligonucleotide therapy targeting microRNA-224 ameliorates pulmonary hypertension by orchestrating the BMP pathway
14:30-15:00	Session 2: Inflammation Therapeutics
	Chair: Ulrike Protzer, TUM, Munich
	14:30-14:50 Stefan Engelhardt, <i>LMU, Munich</i> <u>INV05</u> : Inhalation RNA therapeutics targeted to macrophages
	14:50-15:00 Lea Krutzke, University of Ulm <u>OR03</u> : Extracellular vaccine- or virus-derived SARS-CoV-2 spike protein: a potential link between reported pathologies
15:00-15:40	Wolfram Ostertag Lecture
	Chair: Manuel Grez
	15:00-15:05 Introduction by chair
	15:05-15:40 Dorothee von Laer , <i>Medical University of Innsbruck</i> INV06: A rhabdovirus for oncolysis and immunotherapy of cancer
15:40-16:10	Coffee Break



16:10-17:50	Session 3: Novel concepts in editing
	Chair: Julian Grünewald, TUM Munich
	16:10-16:30 Toni Cathomen , <i>University of Freiburg</i> <u>INV08</u> : New insights in on- and off-target effects of genome editing
	16:30-16:50 Zoltan lvics, <i>PEI, Langen</i> INV09: Next generation Sleeping Beauty transposases for nonviral engineering of therapeutic cells
	16:50-17:10 Frank Buchholz, <i>Dresden University</i> INV10: Engineering Designer-Recombinases for therapeutic genome editing
	17:10-17:30 Angelo Lombardo, <i>SR Tiget, Milan</i> INV11: Exploiting Targeted Epigenome Editing for Therapeutic Applications
	17:30-17:40 Victor Glaser, Charité University Berlin OR04: Enhanced safety in multiplex-edited T cells through combined use of distinct CRISPR enzymes for knock-in and base editing
	17:40-17:50 Sibtain Haider , <i>University Medical Center, Freiburg</i> OR05: Peptide-assisted tethering of DNA repair effectors to Cas9 for precise genome editing
17:50-18:40	Session 4: Keynote
	Chair: Zoltan Ivics, PEI, Langen
	17:50-18:40 Laura Sepp-Lorenzino, Intellia, Boston INV12: Advances in Therapeutic CRISPR Cas9 Applications
18:40-20:00	Afterglow & posterwalk

THURSDAY 7 MARCH

08:30-09:00	Registration
09:00-10:20	Session 5: Editing cardiomyopathies
	Chair: Christian Kupatt, TUM, Munich
	9:00-9:20 Julian Grünewald, <i>TUM, Munich</i> INV13: Engineering CRISPR technologies for application in cardiovascular medicine
	9:20-9:40 Mauro Giacca, <i>Kings College London</i> INV14: RNA Therapies for Cardiac Regeneration and Gene Editing
	9:40-10:00 Simon Lebek , <i>University Clinic Regensburg</i> INV15: CRISPR-Cas9 gene editing as a therapy for common cardiovascular diseases
	10:00-10:20 Charles Gersbach , <i>Duke University</i> <u>INV16</u> : Genome Editing for Duchenne Muscular Dystrophy
10:20-10:50	Coffee Break

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10:50-12:10	Session 6: New preclinical and clinical cancer concepts
	Chair: Roland Rad, TUM, Munich
	10:50-11:10 Claudio Mussolino , <i>University of Freiburg</i> <u>INV17</u> : Multiplexed epigenome editing to protect CAR T cells from cancer-induced activation of inhibitory checkpoints
	11:10-11:30 Liane Preußner, <i>BioNTech, Mainz</i> <u>INV18</u> : BNT211-01: Interim results from a repeat dose escalation study of CLDN6 CAR-T cells manufactured with an automated process ± a CLDN6-encoding CAR-T cell-Amplifying RNA Vaccine (CARVac)
	11:30-11:50 Ben Kleinstiver, Harvard Medical School, Boston INV19: Engineering Improved CRISPR Technologies
	11:50-12:00 Lea-Isabell Schwarze, University Medical Center Hamburg-Eppendorf <u>OR06</u> : Translation of advanced cell therapies for HIV+ patients
	12:00-12:10 Jonas Kath , <i>Charité University Berlin</i> <u>OR07</u> : CD3-zeta gene editing to reprogram T or NK cells with chimeric antigen receptors
12:10-13:40	Lunch & posters
13:40-14:50	Session 7: Cancer cell and virotherapy
	Chair: Dirk Nettelbeck, DFKZ, Heidelberg
	13:40-14:00 Alan Melcher, <i>The Institute of Cancer Research, London</i> <u>INV20</u> : T cell receptor-antigen engagement dynamics with oncolytic virotherapy defines a novel subset of functionally active anti-tumour CD8 cells
	14:00-14:20 Per Sonne Holm , <i>Medical University Innsbruck</i> <u>INV21</u> : Cell cycle inhibition and epigenetic modulation meets YB-1 based virotherapy
	14:20-14:40 Guy Ungerechts, <i>NCT, Heidelberg</i> <u>INV22</u> : Viral Vectors for Cancer Immunotherapy
	14:40-14:50 Frederik Wienen , <i>University of Ulm</i> <u>OR08</u> : An oncolytic HAdV-5 with reduced surface charge combines diminished toxicity and improved tumour targeting
14:50-15:20	Coffee Break
15:20-17:00	Session 8: Novel cell therapeutics
	Chair: Florian Bassermann, TUM, Munich
	15:20-15:40 Andrea Schmidts, <i>TUM, Munich</i> INV23: Engineering new CAR-T platforms
	15:40-16:00 Christine Spitzweg , <i>LMU</i> , <i>Munich</i> <u>INV24</u> : The sodium iodide symporter (NIS) as theranostic gene: its emerging role in new imaging modalities and non-viral gene therapy
	16:00-16:20 Winfried Wels, <i>GSH, Frankfurt</i> INV25: CAR-engineered NK cells: Empowering the first line of defense against cancer
	16:20-16:40 Evelyn Ullrich, <i>Goethe University Frankfurt</i> <u>INV26</u> : Engineering of primary CAR-NK cells paves the way for next generation immunotherapies
	16:40 -16:50 Kerstin Geiger, University of Freiburg OR09: Base editing restore cellular phenotype of T cells of patients with Hyper-IgE-Syndrome
	16:50-17:00 Jiri Eitler , <i>Dresden University of Technology</i> <u>OR10</u> : Dual targeting of PD-L1 and ErbB2 by CAR-NK cells enables specific elimination of solid tumor cells and overcomes immune escape via antigen loss
17:00-18:30	Afterglow & posterwalk
19:00-23:00	Networking event



FRIDAY 8 MARCH

08:30-09:00	Registration
09:00-10:40	Session 9: <i>In vivo</i> gene therapeutics
	Chair: Stefan Kochanek, University of Ulm
	9:00-9:20 Stylianos Michalakis, <i>LMU, Munich</i> <u>INV27</u> : Development of retina-targeted gene therapy: new concepts and remaining challenges
	9:20-9:40 Tarik Bozoglu, <i>TUM, Munich</i> <u>INV28</u> : Affinity peptide mediated retargeting of AAV9 to cardiac interstitial cells
	9:40-10:00 Hildegard Büning, MHH, Hannover INV29: Tailoring Adeno-associated virus (AAV) vectors for in vivo gene therapy
	10:00-10:20 Dirk Grimm, <i>University of Heidelberg</i> <u>INV30</u> : AAV (finally) flexes its muscles: Evolution and application of myotropic AAV capsid variants
	10:20-10:30 Marco Radukic, <i>Bielefeld University</i> OR11: ITR instability in E. coli? The answer is 42 (°C) for improving quantity and quality of rAAV
	10:30-10:40 Ceren Kimna, <i>Helmholtz Center Munich</i> OR12: Cell Level Imaging of Nucleic Acid Therapeutics in Whole Mouse Bodies
10:40-11:10	Coffee Break
11:10-11:20	Session 10: Introduction of the National Network Office for Gene and Cell Therapies
	11:10-11:20 Elke Luger , <i>National Network Office for Gene and Cell Therapy</i> The National Network for Gene and Cell Therapies – just another network?
11:20-12:35	Session 11: Young investigator session
	Chair: Hildegard Büning, MHH, Hannover
	11:20-11:35 Daniela Paasch , <i>MHH, Hannover</i> <u>OR13:</u> Scalable generation of functional human iPSC-derived CAR-macrophages that efficiently eradicate CD19- positive leukemia
	11:35-11:50 Samuel Theuerkauf , <i>Paul Ehrlich Institute, Langen</i> <u>OR14</u> : AAV vectors displaying bispecific DARPins enable dual-control targeted gene delivery
	11:50- 12:05 Juliane Schott, <i>MHH, Hannover</i> <u>OR15:</u> Third-generation lentiviral gene therapy rescues function in a mouse model of Usher 1B
	12:05-12:20 Martin Bentler , <i>MHH, Hannover</i> <u>OR16</u> : Modifying immune responses to adeno-associate virus vectors by capsid engineering
	12:20-12:35 Max Wichmann, University Medical Center Hamburg-Eppendorf, Hamburg OR17: Deep characterization and comparison of different retrovirus-like particles preloaded with CRISPR/Cas9 RNPs
12:35-13:35	Session 12: Ethical considerations
	Chair: Christopher Baum, Berlin Institute of Health
	12:35-12:55 Klaus Tanner, University of Heidelberg INV31: Ethics through bureaucracy? The limits of formal rationality
	12:55-13:15 Jens Kersten, <i>LMU, Munich</i> INV32: A strategy of obsolete law?
	13:15-13:35 Gerd Maass , <i>Roche, Munich</i> INV33: Challenges and opportunities of CGT - the industrial perspective



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