

1ST ADVANCED THERAPIES SCIENCE MEETING

25-26 November 2019 | Maritim Hotel , Berlin



An application has been made to the EACCME® for CME accreditation of this event

Monday, 25 November 2019- Day 1

08.00-9.00 Registration

Welcome
Plenary Room

09.00-09.05 Welcome
Hans-Dieter Volk, Petra Reinke

Plenary Room

09.05-09.40 Introduction

RESTORE – Health by Advanced Therapies – Opportunities and Challenges
Hans-Dieter Volk

Opening Remarks of Ada Yonath, Nobel Prize for Chemistry
Director of Weizmann Institute of Science, Israel

Session 1 Foundational Research (new targets and new indications)

Chair: to be added

Plenary Room

09.40-10.05 On the way to improved therapies for skin diseases and disorders (20 + 5 min discussion)
Alexandra Marques - University of Minho, Portugal

10.05-10.20 Targeting the tumour vasculature with CAR-T cells for treatment of solid tumours (10 + 5 min discussion)
Andrea Keogh- Catapult, United Kingdom

10.20-10.50 Coffee Break & Posters set up

10.50-11.05 Characterizing immune cells by Single-Cell-Sequencing - Identification of novel therapeutic targets for precision cell therapy in chronic inflammation (10 + 5 min discussion)
Mir-Farzin Mashreghi- DRFZ, Germany

11.05-11.20 Bioengineered implants to repair the heart (10 + 5 min discussion)
Maximilian Emmert- University Zurich, Switzerland

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11.20-11.35 Immunobiology of CarT cells and adoptive T cell therapy of cancer and autoimmune diseases (10 + 5 min discussion)
Maksim Mamonkin-affiliation to be added

11.35-11.45 Break

Session 2 Preclinical Models and Technologies (focus on human-on-a-chip)

Chair: to be added

Plenary Room

11.45-12.10 Engineered polymeric matrices as instructive cells' artificial micro-environment (20+ 5 min discussion)
Antonella Motta - University of Trento, Italy

12.10-12.45 Stimuli-responsive and multifunctional scaffold-cells systems for tendon tissue regeneration (20+ 5 min discussion)
Manuela Gomes - University of Minho, Portugal

12.45-13.00 From Organ-on-a-Chip Tools Towards Patients on Chips – Enforcing a Paradigm Shift in Drug Development (10 + 5 min discussion)
Uwe Marx- TissUse, Germany

13.00-13.15 Monitoring CAR T cell efficacy in fresh human solid tumor slices (10 + 5 min discussion)
Emmanuel Donnadieu- Inserm, France

13.15-13.20 Break

13.20-13.30 Photo session

13.30-14.30 Lunch & poster setup finalization

Session 3 Manufacturing Technologies (including product characterisation and automation)

Chair: to be added

Plenary Room

14.30-14.55 Engineering personalized tissue implants: From 3D printing to bionic tissues (20+ 5 min discussion)
Tal Dvir - Tel Aviv University, Israel

14.55-15.20 Genome-wide CRISPR Screens in Primary Human T Cells Reveal Key Regulators of Immune Function (20+ 5 min discussion)

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Eric Shifrut - University of California, San Francisco, United States

- 15.20-15.35 **Becoming cell therapy Makers - Opportunities and challenges in manufacturing of cell therapy products** (10 + 5 min discussion)
Lior Raviv - Pluristem, Israel
- 15.35-15.50 **Pioneering Cell Processing of personalized ATMPs** (10 + 5 min discussion)
Michael Apel - Miltenyi, Germany
- 15.50-16.05 **Generation of hematopoietic cells with lymphoid potential from induced pluripotent stem cells (iPSC) for adoptive immunotherapy of cancer and leukemia** (10 + 5 min discussion)
Annelise Bennaceur - INSERM, France
- 16.05-16.20 **Functional Surfaces and Scaffolds Combined with Stem Cells for Bone and Cartilage Tissue Engineering** (10 + 5 min discussion)
Nuno Neves - University Minho, Portugal
- 16.20-16.30 **Break**

Break out sessions in Plenary and break-out rooms for topics 1-3 (10 minutes each)

1 Foundational Research (new targets and new indications) (Plenary)

- 16.30-16.40 **Developing Novel siRNA Nanoparticle Therapies For Cystic Fibrosis by Silencing of the Epithelial Sodium Channel**
Stephen Hart - UCL Great Ormond Street Institute of Child Health, UK
- 16.40-16.50 **Epigenetic control of T cell differentiation: From genome-wide signatures to local regulators and their targeted manipulation.**
Julia K. Polansky - BCRT Charité, Germany
- 16.50-17.00 **Subconjunctival injection of low-dose mesenchymal stem cells promotes corneal allograft survival in a mouse cornea transplantation model**
Thomas Ritter - National University of Ireland Galway, Ireland
- 17.00-17.10 **Immunomodulatory properties of amniotic membrane derivatives for tissue regeneration: a 20-year experience**
Ornella Parolini - Università Cattolica Santo Cuore, Italy
- 17.10-17.40 **Coffee Break**
- 17.40-17.50 **Pre-activated mesenchymal stromal cells induce regulatory immune populations in vivo and prolong corneal allograft survival**
Thomas Ritter - National University of Ireland Galway, Ireland
- 17.50-18.00 **Treatment of osteochondral defects of the knee in rats using bilayered scaffold free cell-based constructs**

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Luis Freitas Mendes - KULEUVEN, Belgium

- 18.00-18.10 **Highly activated Natural Killer cells to treat pediatric cancer**
Maria Vela - Hospital La Paz Institute for Health Research (IdiPAZ), Spain
- 18.10-18.20 **Neural precursor/stem cell-based therapy for Rett syndrome**
Nicoletta Landsberger - University of Milan, Italy
- 18.20-18.30 **Drive regeneration by employing the immune-structure interface**
Georg Duda - BCTR Charité, Germany

2 Preclinical Models and Technologies (focus on human-on-a-chip) (Berlin D or London)

- 16.30-16.40 **Tissue engineered mimetic autografts for bone regeneration**
Froilán Granero-Moltó - Clínica Universidad de Navarra, Spain
- 16.40-16.50 **A millifluidic dynamic system to unveil the interaction among breast cancer cells, fibroblasts and mesenchymal stem cells**
Virginia Brancato, University of Minho, Portugal
- 16.50-17.00 **Aryl hydrocarbon receptor- driven immunometabolic checkpoints in type-1 dendritic cells restrains antitumor responses**
Francesca Fallarino - Università di Perugia, Italy
- 17.00-17.10 **Amniotic mesenchymal stromal cell secretome: a potent pleiotropic strategy for acute brain injury**
Francesca Pischiutta - Istituto di Ricerche Farmacologiche Mario Negri IRCCS, Italy
- 17.10-17.40 **Coffee Break**
- 17.40-17.50 **Immunosuppressant-resistant T cells for advanced adoptive T cell therapy in immunosuppressed patients**
Laila Amini, BCRT- Charité, Germany
- 17.50-18.00 **Adapter CAR T cells - A new versatile platform for controllable CAR T cell function**
Joerg Mittelstaet, Miltenyi Biotec GmbH, Germany
- 18.00-18.10 **Microvessels-on-Chip - Bridging the gap between blood and lymph**
Barbara Bachmann, Ludwig Boltzmann Institute for Experimental and Clinical Traumatology and Vienna University of Technology, Austria
- 18.10-18.20 **MSC improve skeletal muscle regeneration via locally dampen the pro-inflammatory capacity of CD8+ T cell subpopulations**
Sven Geissler, BCRT Charité, Germany
- 18.20-18.30 **Treatment of a naturally occurring dog model of Alport Syndrome by means of CRISPR/Cas9 gene therapy approach.**
Sergio Daga, University of Siena, Medical Genetics, Italy

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- 18.30-18.40 **Effectiveness and safety of adoptive cell therapy with regulatory type 1 (Tr1) cells in pancreatic islet transplantation**
Georgia Fousteri, San Raffaele Diabetes Research Institute, Italy
- 18.40-18.50 **A preclinical non-human primate model to investigate the regenerative potential of human induced pluripotent stem cell-derived cardiomyocytes**
Ina Gruh, Hannover Medical School, Germany

3 Manufacturing Technologies (including product characterisation and automation) (Berlin D or London)

- 16.30-16.40 **Tissue Manufacturing by Bioprinting: Challenges and Opportunities for Regenerative Medicine**
Fabien Guillemot - Poietis, France
- 16.40-16.50 **Self-assembly of stem/progenitor cells creates human neo-vascularized skin and skin organoids**
Patricia Peking - Paracelsus Medical University, Austria
- 16.50-17.00 **Using clinical data for manufacturing design and release criteria to improve the quality of a cell-based ATMP for cartilage repair**
Giulietta Roel, - CO.DON AG, Germany
- 17.00-17.10 **Harmonisation of multicenter MSC production for a phase III clinical trial**
Melissa Van Pel - Leiden University Medical Center, Netherlands
- 17.10-17.40 **Coffee Break**
- 17.40-17.50 **Manufacturing of stem cell derived in-vitro tissues using robot technology**
Marco Metzger - Fraunhofer Institute for Silicate Research (ISC), Germany
- 17.50-18.00 **EU Project CARAT: Chimeric Antigen Receptors (CARs) for Advanced Therapies**
Michael Apel, - Miltenyi Biotec GmbH, Germany
- 18.00-18.10 **Human induced pluripotent stem cells from universal donors as starting material for regenerative therapies**
Micha Drukker, Helmholtz Center Munich, Germany
- 18.10-18.20 **Gene therapy getting personal: mutation-specific editing and gene addition strategies**
Carsten Werner Lederer, The Cyprus Institute of Neurology and Genetics / TEDDY, Cyprus
- 18.20-18.30 **T cell immunity toward CRISPR-associated nucleases**
Dimitrios Laurin Wagner, Charité, Germany
- 18.30-18.40 **Tissue-Engineered ATMPs for cartilage healing**
Oliver Pullig, Fraunhofer Translational Center Regenerative Therapies - TLC-RT, Germany

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- 18.40-18.50 Decentralised manufacturing using the automated CliniMACS Prodigy platform for a multicenter clinical trial in Germany
Michael Apel, Miltenyi Biotec GmbH, Germany
- 18.50-19.00 Personalized therapy for TP53 mutated cancer patients based on CRISPR-Cpf1 and suicide gene delivery
Filomena T. Papa, University of Siena, Italy
- 19.00-19.10 Generation, cultivation and characterization of stem cell-derived bioartificial cardiac tissue of clinically relevant dimensions
Ina Gruh, Hannover Medical School, Germany

Poster session & Networking Get-Together

- 19.30-21.00 Poster session & discussion with Cheese & Wine buffet
- 21.00-22.00 Poster session continued

Tuesday, 26 November 2019- Day 2

Introduction Day 2

Plenary Room

- 08.30-08.45 Registration for arrival day 2
- 08.45-09.00 *Hans-Dieter Volk (15 min)*

Session 4 Clinical Implementations (including reimbursement models)

Chair: to be added

Plenary Room

- 09.00-09.25 Epithelial stem cell-mediated gene therapy (20 + 5 min discussion)
Michele De Luca - University of Modena, Italy
- 09.25-09.50 Clinical implementation of cornea and skin artificial tissues generated with fibrin-agarose biomaterials (20 + 5 min discussion)
Antonio Campos - University of Granada, Spain
- 09.50-10.15 Improving the expansion and persistence of tumor-specific T-cells targeting immunosuppressive tumors (20 + 5 min discussion)
Cliona M. Rooney - Baylor College of Medicine, Houston, United States

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- 10.15-10.30 **Reshape immune balance by next-generation regulatory T cells (10 + 5 min discussion)**
Petra Reinke - BeCAT Charité Germany
- 10.30-10.45 **Cellular therapy: Boosting innovation in technology and clinical translation (10 + 5 min discussion)**
Michael Apel - Miltenyi Biotech GmbH Germany
- 10.45-11.00 **Development of allogeneic Placenta-derived (PLX) cell therapy- from bench- to bedside (10 + 5 min discussion)**
Racheli Ofir - Pluristem, Israel
- 11.00-11.15 **Car-T cell therapy- a clinical experience report (10 + 5 min discussion)**
Michal Besser- affiliation to be added
- 11.15 -12.00 **Coffee Break**

Session 5 Regulatory Science & Clinical Trials

Chair:

Plenary Room

- 12.00-12.25 **Role of the EMA Committee for Advance Therapies (CAT) in the assesment of ATMPs (20+ 5 min discussion)**
Paolo Gasparini - University of Trieste, IRCCS, Italy
- 12.25-12.50 **MSCs as a therapy for diabetic complications: a translational journey in the EU (20+ 5 min discussion)**
Timothy O'Brien
- 12.50-13.05 **An Integrated Eco-System of Research and Development for Orphan Medicinal Products: the IRDiRC Orphan Drug Development Guidebook (10+ 5 min discussion)**
Michela Gabaldo - Fondazione Telethon, Italy
- 13.05-13.10 **Break**

13.10-14.15 Lunch

Session 6 Big data- AI-Ethics Economics

Chair:

Plenary Room

- 14.15-14.30 **State and potential for data-driven strategies in the cell and gene therapy industry (10+ 5 min discussion)**
Marc-Olivier Baradez - Catapult, United Kingdom

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- 14.30-14.45 **Ethics management of science and the public** (10+ 5 min discussion)
Andreas Kurtz - Charité, Germany
- 14.45-15.00 **Ethics in scientific communication** (10+ 5 min discussion)
Pier- Maria Fornasari
- 15.00-15.15 **A Consistent Mechanism for Designing a Clinical Trial based on AI Technologies Applied to Observational Cohort** (10 + 5 min discussion)
Michal Rosen-Zvi - IBM Research, Israel

Short talks

- 15.15-15.25 **In silico regenerative medicine: from living implants to digital patients and back again** (8 + 2 min discussion)
Liesbeth Geris - KU Leuven, Belgium
- 15.25-15.35 **Stem cell data standards and data utility** (8 + 2 min discussion)
Andreas Kurtz - Charité, Germany
- 15.35-15.45 **Break**

Break out sessions in Plenary and break-out rooms for topics 4-5 (10 minutes each)

4 Clinical Implementations (including reimbursement models) (Plenary)

- 15.45-15.55 **Stem cells for treatment of Amyotrophic lateral sclerosis.**
Eva Sykova - Scimed Biotechnologies, Czech Republic
- 15.55-16.05 **Chimeric antigen receptor (CAR) immunotherapy for patients with solid tumors**
Gabriele Pecher - Charité, Germany
- 16.05-16.15 **SLAMF7 CAR-T cells for immunotherapy of multiple myeloma: 'real-world' experience of GMP-manufacturing using virus-free Sleeping Beauty gene-transfer**
Michael Hudecek - Universitätsklinikum Würzburg, Germany
- 16.15-16.25 **Cartilage regeneration with ATMP produced cells; past, present and future perspectives**
Anders Lindahl - Institute of Biomedicine, University of Gothenburg, Sweden
- 16.25-16.35 **Adoptive Transfer of Thymic Derived Regulatory T-Cells in Therapy-Refractory Chronic Graft versus Host Disease in Children**
Sybille Landwehr-Kenzel, Charité, Germany

5 Regulatory Science & Clinical Trials (Berlin D or London)

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- 15.45-15.55 Technology Maturation Platform, Public-Private Partnerships, bench-to-bedside translational development
Roke Iñaki Oruezabal - Andalusian Network for Design and Translation of Advanced Therapies, Spain
- 15.55-16.05 Translation of CAR-based innovation into clinics: how to overcome regulatory hurdles
Michael Apel, - Miltenyi Biotec GmbH, Germany
- 16.05-16.15 Personalized tissue-engineered grafts
Raimund Strehl - Verigraft AB, Sweden
- 16.15 -16.25 Placental Cell Therapy for the Treatment of Muscle Trauma: From Preclinical Models to Clinical Phase III
Tobias Winkler - Charité, Germany
- 16.40-17.40 Poster session & Coffee Break
- Final Remarks, Farewell (Plenary)**
- 17.40-18:00 Final Remarks, Farewell
- 18:00 End of meeting