



Agenda

1st ADVANCED THERAPIES SCIENCE MEETING

25th-26th November 2019 - Berlin, Germany



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Health by Advanced Therapies

Monday, 25th November 2019 - Day I

08.00-09.00 Registration

Welcome

Hall Maritim

09.00-09.05 **Welcome**

Hans-Dieter Volk - Charité Universitätsmedizin Berlin, Germany

Petra Reinke - BeCAT - Charité Universitätsmedizin Berlin, Germany

09.05-09.25 **Introduction**

RESTORE - Health by Advanced Therapies - Opportunities and Challenges

Hans-Dieter Volk - Charité Universitätsmedizin Berlin, Germany

09.25-09.40 **Opportunities of Advanced Therapies - an Israel view**

Ami Appelbaum - Israel Innovation Authority, Israel

Plenary Session Topic 1 - Foundational Research (New Targets and New Indications)

Hall Maritim

Chairs: Annelise Bennaceur - Institute National de la Santé et de la Recherche, France

Georg Duda - BCRT, Charité Universitätsmedizin Berlin, Germany

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 - Cell and Gene Therapy Catapult, United Kingdom

10.20-10.45 *Coffee break & Posters set up*

10.45-11.00 **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 - Deutsches Rheuma-Forschungszentrum, Germany

11.00-11.15 **Bioengineered implants to repair the heart** (10 + 5 min discussion)

Maximilian Emmert - University of Zurich, Switzerland

11.15-11.40 **Engineering T-cells to target malignant and pathogenic T-cells** (20 + 5 min discussion)

Maksim Mamonkin - Baylor College of Medicine, United States

Plenary Session Topic 2 - Preclinical Models and Technologies (Focus on Human-on-a-chip)

Hall Maritim

Chairs: Ali Turhan - Institute National de la Santé et de la Recherche, France
Uwe Marx - TissUse GmbH, Germany

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

12.10-12.35 **Stimuli-responsive and multifunctional scaffold-cells systems for tendon tissue regeneration**
(20 + 5 min discussion)
Manuela Gomes - University of Minho , Portugal

12.35-12.50 **From Organ-on-a-Chip Tools Towards Patients on Chips - Enforcing a Paradigm Shift in Drug Development** (10 + 5 min discussion)
Uwe Marx - TissUse GmbH, Germany

12.50-13.05 **Monitoring CAR T cell efficacy in fresh human solid tumor slices** (10 + 5 min discussion)
Emmanuel Donnadieu - Institute National de la Santé et de la Recherche, France

13.15-13.30 **Photo session**

13.30-14.30 *Lunch & Poster setup finalisation*

Plenary Session Topic 3 - Manufacturing Technologies (Including Product Characterisation and Automation)

Hall Maritim

Chairs: Sebastian Knöbel - Miltenyi Biotec GmbH, Germany
Rui Reis - University of Minho, Portugal

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)
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 LTD, Israel

15.35-15.50 **Pioneering Cell Processing of personalized ATMPs** (10 + 5 min discussion)
Andrew Kaiser - Miltenyi Biotec GmbH, 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 - Institute National de la Santé et de la Recherche, 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 of Minho, Portugal

Break out sessions in Berlin D Room, Hall Maritim and London Room for topics 1-3 (in parallel)

Break out Session Topic 1 - Foundational Research (New Targets and New Indications)

Berlin D Room

Chair: Andreas Bosio - Miltenyi Biotech GmbH, Germany

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, United Kingdom

16.40-16.50 **Epigenetic control of T cell differentiation: From genome-wide signatures to local regulators and their targeted manipulation.**
Julia K. Polansky-Biskup - BCRT, Charité Universitätsmedizin Berlin, 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**
Oliver Treacy - 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**
Luis Freitas Mendes - Katholieke Universiteit Leuven, Belgium

18.00-18.10 **Highly activated Natural Killer cells to treat pediatric cancer**
Cristina Ferreras - Hospital La Paz Institute for Health Research, 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 - BCRT, Charité Universitätsmedizin Berlin, Germany

18.30-18.40 **Towards efficient strategies of HIV-1 cure: Analysis of HIV-1 latency-reverting agents by single cell RNA-seq**
Christine Laqmani-Goffinet - Charité Universitätsmedizin Berlin, Germany

Break out Session Topic 2 - Preclinical Models and Technologies (Focus on Human-on-a-chip)

Hall Maritim

Chair: Nuno Neves - University of Minho, Portugal

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 - University of 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 **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

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 Universitätsmedizin Berlin, 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, Italy

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 **Immunosuppressant-resistant T cells for advanced adoptive T cell therapy in immunosuppressed patients**

Leila Amini - BCRT, Charité Universitätsmedizin Berlin, Germany

Break out Session Topic 3 - Manufacturing Technologies (Including Product Characterisation and Automation)

London Room

Chair: Michael Schmück-Henneresse - BCRT, Charité Universitätsmedizin Berlin, Germany

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, Germany

17.50-18.00 **Human induced pluripotent stem cells from universal donors as starting material for regenerative therapies**
Micha Drukker - Helmholtz Center Munich, Germany

18.00-18.10 **Gene therapy getting personal: mutation-specific editing and gene addition strategies**
Carsten Werner Lederer - The Cyprus Institute of Neurology and Genetics / TEDDY, Cyprus

18.10-18.20 **T cell immunity toward CRISPR-associated nucleases**
Dimitrios Laurin Wagner - BCRT, Charité Universitätsmedizin Berlin, Germany

18.20-18.30 **Tissue-Engineered ATMPs for cartilage healing**
Oliver Pullig - Fraunhofer Translational Center Regenerative Therapies, Germany

18.30-18.40 **Personalized therapy for TP53 mutated cancer patients based on CRISPR-Cpf1 and suicide gene delivery**
Flaminia C. Lorenzetti - University of Siena, Italy

18.40-18.50 **Generation, cultivation and characterization of stem cell-derived bioartificial cardiac tissue of clinically relevant dimensions**
Ina Gruh - Hannover Medical School, Germany

18.50-19.10 **Discussion**

Poster session & Networking Get-Together

19.30-21.00 **Poster session & Discussion** *with Cheese & Wine buffet*

21.00-22.00 **Poster session continued**

Tuesday, 26th November 2019 - Day II

Introduction Day II

Hall Maritim

08.30-08.45 Registration

08.45-09.00 **Introduction** (15 min)

Hans-Dieter Volk - Charité Universitätsmedizin Berlin, Germany

09.00-09.15 **Opening Remarks of Ada Yonath, Nobel Prize for Chemistry** (15 min)

Ada Yonath - Director of Weizmann Institute of Science, Israel

Plenary Session Topic 4 - Clinical Implementations (Including Reimbursement Models)

Hall Maritim

Chairs: Zami Aberman - Pluristem LTD, Israel

Terri Gaskell - Cell and Gene Therapy Catapult, United Kingdom

09.15-09.40 **Combined cell and gene therapy for Epidermolysis Bullosa** (20 + 5 min discussion)

Michele De Luca - University of Modena, Italy

09.40-10.05 **Clinical implementation of cornea and skin artificial tissues generated with fibrin-agarose biomaterials** (20 + 5 min discussion)

Antonio Campos - University of Granada, Spain

10.05-10.30 **Car-T cell therapy - a clinical experience report** (20 + 5 min discussion)

Michal Besser - Ella Lemelbaum Institute of Immuno-Oncology Sheba Medical Center, Israel

10.30-10.45 **Reshape immune balance by next-generation regulatory T cells** (10 + 5 min discussion)

Petra Reinke - BeCAT - Charité Universitätsmedizin Berlin, Germany

10.45-11.00 **Cellular therapy: Boosting innovation in technology and clinical translation** (10 + 5 min discussion)

Mario Assenmacher - Miltenyi Biotech GmbH, Germany

11.00-11.15 **Development of allogeneic Placenta-derived (PLX) cell therapy - from bench - to bedside** (10 + 5 min discussion)

Racheli Ofir - Pluristem LTD, Israel

11.15-11.50

Coffee break

Plenary Session Topic 5 - Regulatory Science and Clinical Trials

Hall Maritim

Chairs: Petra Reinke - BeCAT - Charité Universitätsmedizin Berlin, Germany
Racheli Ofir - Pluristem LTD, Israel

- 11.50-12.15 **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
- 12.15-12.40 **MSCs as a therapy for diabetic complications: a translational journey in the EU** (20 + 5 min discussion)
Timothy O'Brien - National University of Ireland, Ireland
- 12.40-12.55 **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
- 13.00-14.15 *Lunch*

Plenary Session Topic 6 - Special Workshop - Ethics, Economics, Big data and AI

Hall Maritim

Chairs: Joana Namorado - IBMT Fraunhofer Institut, Germany
Michal Rosen-Zvi - IBM Research, Israel

- 14.15-14.30 **State and potential for data-driven strategies in the cell and gene therapy industry**
(10 + 5 min discussion)
Matthias Fastenrath - Cell and Gene Therapy Catapult, United Kingdom
- 14.30-14.45 **Ethics management of science and the public** (10 + 5 min discussion)
Andreas Kurtz - BCRT, Charité Universitätsmedizin Berlin, Germany
- 14.45-15.00 **Ethics in scientific communication** (10 + 5 min discussion)
Pier Maria Fornasari - Regen Health Solutions, Italy
- 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
- 15.15-15.25 **In silico regenerative medicine: from living implants to digital patients and back again**
(8 + 2 min discussion)
Liesbet Geris - Katholieke Universiteit Leuven, Belgium
- 15.25-15.35 **Stem cell data standards and data utility** (8 + 2 min discussion)
Andreas Kurtz - BCRT, Charité Universitätsmedizin Berlin, Germany

Break out sessions in Hall Maritim and Berlin D Room for topics 4-5 (in parallel)

Break out Session Topic 4 - Clinical Implementations (Including Reimbursement Models)

Hall Maritim

Chair: Mohamed Abou EL-Enein - BCRT, Charité Universitätsmedizin Berlin, Germany

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 Universitätsmedizin Berlin, 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 - 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 - BCRT, Charité Universitätsmedizin Berlin, Germany

Break out Session Topic 5 - Regulatory Science and Clinical Trials

Berlin D Room

Chair: Michela Gabaldo - Fondazione Telethon, Italy

15.45-15.55 **Translational support in tissue engineering by the Andalusian Network for Design and Translation of Advanced Therapies**

Rosario Sánchez Pernaute - 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**

Bernd Schröder - 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 - BCRT, Charité Universitätsmedizin Berlin, Germany

16.40-17.40 **Poster session & Coffee break**

Final Remarks, Farewell

Hall Maritim

17.40-18.00 **Final Remarks, Farewell**

Hans-Dieter Volk - Charité Universitätsmedizin Berlin, Germany

Scientific Committee

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RESTORE

Health by Advanced Therapies



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