



International Center
for Scientific Debate
BARCELONA



ADVANCED CELLULAR THERAPIES AND REGENERATIVE MEDICINE

THE PROMISE IN THE 21ST CENTURY

February, 17th, 2015 COSMOCAIXA BARCELONA. C/ Isaac Newton, 26. Barcelona
February, 18th, 2015 BANC DE SANG I TEIXITS. Passeig Taulat, 116. Barcelona

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ADVANCED CELLULAR THERAPIES AND REGENERATIVE MEDICINE

THE PROMISE IN THE 21ST CENTURY

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ADVANCED CELLULAR THERAPIES AND REGENERATIVE MEDICINE

THE PROMISE IN THE 21ST CENTURY

February, 17th and 18th, 2015

WELCOME

Dear Speakers and Participants,

It is a pleasure and an honor to welcome you to the scientific meeting “Advanced Therapies and Regenerative Medicine. The promise in the 21st Century”, co-organized by the B·DEBATE International Center for Scientific Debate Barcelona (an initiative of Biocat with support from “la Caixa” Foundation), and Banc de Sang i Teixits (BST) with the support of TerCel, the Spanish Cell Therapy Network and the International Society for Cellular Therapy (ISCT).

Advanced Therapies and Regenerative Medicine are two tightly linked emerging areas that will likely be an essential part of the future of medical care. Both are contributing to the building of a new paradigm: the cure of diseases by using cells, tissues, molecules and other components aiming to the restitution of the lost functions and properties of diseased cell, tissues or, eventually, organs.

Among decades, thousands of investigators have attempted to crack the intimate code of cell regulation and biology of development. Today, the results of their research are reaching clinical application. A number of clinical trials are being conducted worldwide and some products are in advanced process of development or (a few) already registered.

Although there are a number of patients already cured by cellular therapies and, more specifically by Advanced Therapies Medicinal Products (ATMs), the scientific community is aware that the road to their fully established clinical application is rather long and fraught with uncertainties. Moreover, research results frequently raise new questions and new research objectives that continuously push investigators and institutions to persevere on their effort and investment in this area of science.

Some of these still open questions will be the target of our meeting which goal is to bring together many of the experts in the field to have an open and free debate and, if possible, to reach conclusions or key messages useful for the scientific development and ultimately for patients benefit.

We encourage you to actively participate in the interesting discussions over the next two days.

Yours, sincerely

Joan Garcia, Scientific Leader of the event, and **B·Debate**

PROGRAM

Tuesday, February, 17th, 2015

COSMOCAIXA BARCELONA. C/ ISAAC NEWTON, 26 . BARCELONA

9:00 **Welcome**

9:15 **KEYNOTE SPEAKERS: History of Cellular Therapies, Achievements, Challenges Faced and Milestones for the Future**

Alejandro Madrigal, Anthony Nolan Foundation, London, UK

Sergi Querol, Banc de Sang i Teixits (BST), Barcelona, Spain

10:00 **SESSION 1. TISSUE ENGINEERING AND REGENERATION. REAL CASES AND STANDPOINTS**

Chairs: **Alejandro Madrigal**, Anthony Nolan Foundation, London, UK

Anna Velga, Centre de Medicina Regenerativa de Barcelona, Barcelona, Spain

10:15 **Regenerative Medicine Therapies in the 21st Century**

Julie Allickson, Wake Forest Institute for Regenerative Medicine, Winston, NC, USA

10:35 **Development and Clinical Use of Bio-engineered Skin**

José Luis Jorcano, CIEMAT, Madrid, Spain

10:55 **Coffee Break**

11:20 **Treatment of Chronic Arthritis with Adipose Tissue Stromal Vascular Fraction**

Jaroslav Michalek, Masaryk University, Brno, Czech Republic

11:40 **Open Debate**

12:40 **Position Statement of the International Society for Cellular Therapy (ISCT)**

Massimo Dominici, Laboratory of Cell Biology and Advanced Cancer Therapy, University of Modena and Reggio Emilia, Italy

13:00 **Lunch**

14:00 **SESSION 2. WHICH CELLS? FIVE SOURCES TO CHOOSE FROM**

Chairs: **Agustin Zapata**, UCM, Madrid, Spain

Massimo Dominici, Laboratory of Cell Biology and Advanced Cancer Therapy, University of Modena and Reggio Emilia, Italy

14:15 **Biological Properties and Clinical Grade Production Development of Bone Marrow Mesenchymal Stromal Cells**

Joaquim Vives, XCELIA, Advanced Therapies Division. Banc de Sang i Teixits, Barcelona, Spain

14:35 **Adipose Tissue Mesenchymal Stromal Cells Properties and Clinical Application**

Damián García-Olmo, IDCsalud Fundación Jiménez Díaz, Madrid, Spain

14:55 **Cellular Bone Matrix, a Differentiated Cellular Allograft for the Repair or Reconstruction of Musculoskeletal Defects**

Alan Smith, LifeNet Health, Virginia Beach, USA

15:15 **T Cell Immunotherapy for Infectious Diseases**

Hermann Einsele, Würzburg University Medical Center, Würzburg, Germany

15:35 **Induced Pluripotent Stem (IPS) Cells for Disease Modeling and Treatment**

Ángel Raya, Centre de Medicina Regenerativa de Barcelona, Barcelona, Spain

15:55 **Open Debate**

16:30 **Coffee Break**

PROGRAM

Tuesday, February, 17th, 2015

COSMOCAIXA BARCELONA. C/ ISAAC NEWTON, 26 . BARCELONA

16:45 SESSION 3. FOR WHAT DISEASES? WHO CAN BENEFIT FROM CELLULAR THERAPY NOW AND IN THE NEAR FUTURE?

Chairs: **Francisco Maculé**, Hospital Clínic, Barcelona, Spain

Damián García-Olmo, IDCsalud Fundación Jiménez Díaz, Madrid, Spain

17:00 Heart Diseases: Who Can benefit from Cellular Therapy Now and in the Near Future?

Manuel Gallñanes, Hospital Vall d'Hebron, Barcelona, Spain

17:20 Diabetes Mellitus. How Can Be Treated by Cellular Medicines?

Bernat Soria, CABIMER, Sevilla, Spain

17:40 Mesenchymal Stem Cells for Multiple Sclerosis: Hypes and Hopes

Antonio Uccelli, University of Genoa, Italy

18:00 Bone and Cartilage Regeneration with Cells and Tissue Engineering Products

Enric Càceres, European Federation of National Associations of Orthopaedic and Traumatology, UAB, Barcelona, Spain

18:20 Cellular Immunotherapies for Hematological Malignancies

Javier Briones, Hospital de Sant Pau, Barcelona, Spain

18:40 Open Debate

19:30 Cocktail at CosmoCaixa

PROGRAM

Wednesday, February, 18th, 2015

BANC DE SANG I TEIXITS. PASSEIG TAULAT, 116. BARCELONA

9:30 SUMMARY OF THE SESSIONS HELD THE DAY BEFORE

Elena Hernández, Banc de Sang i Teixits, Barcelona, Spain

Aurora Masip, Banc de Sang i Teixits, Barcelona, Spain

10:00 Visit to the Blood and Tissue Bank Facilities

Lluís Pulg, Banc de Sang i Teixits, Barcelona, Spain

10:30 Coffee break

11:00 OPEN SESSION: FROM BENCH TO THE BEDSIDE

Chair: **José María Moraleda**, TerCel & Hospital Clínico Universidad Virgen de la Arrixaca, Murcia, Spain

11:10 Advanced Therapies Medicinal Products (ATMs) Regulations in the EU and Spain

Marcos Timón, Agencia Española del Medicamento y Productos Sanitarios, Madrid, Spain

11:50 Relevance of Pre-Clinical Development. The Catapult Experience

Nina Bauer, Catapult, London, UK

12:30 The Tigenix Case: the First Company Having a EMA Registered ATM

Eduardo Bravo, Tigenix, Madrid, Spain

13:10 Concluding Remarks

Joan Garcia, XCELIA Advanced Therapies Division, Banc de Sang i Teixits, Barcelona, Spain

José María Moraleda, TerCel & Hospital Clínico Universidad Virgen de la Arrixaca, Murcia, Spain

AIM OF THE DEBATES

During the next two days, we'll have the opportunity to debate on the present and future of Advanced Therapies and Regenerative Medicine.

We have to admit that it is a quite wide topic and the design of the program addressing **transversal questions**, where different medical specialties and indications are mixed, is rather challenging. But, precisely, our aim has been to debate on the common issues affecting the different approaches of curing diseases by using cells and tissue engineering products.

Here, I have taken the freedom to bring a piece of the introduction of the *2013 Annual Report of the Alliance of Regenerative Medicine*, pointing out the medical and social significance of this scientific field.

- *Regenerative medicine represents a new paradigm in human health with the potential to resolve unmet medical needs by addressing the underlying causes of disease.*
- *The emerging field of regenerative medicine is unique in its aim to augment, repair, replace or regenerate organs and tissue that have been damaged by disease, injury or even the natural aging process. This rapidly evolving, interdisciplinary field is transforming healthcare by translating fundamental science into a variety of regenerative technologies including biologics, chemical compounds, materials and devices. It differs from other fields of medicine in the array of disciplines it brings together and in its ability to create or harness the body's innate healing capacity.*
- *Why is Regenerative Medicine Important to the Future of Healthcare?*
- *Currently, the vast majority of treatments for chronic and/or life-threatening diseases are palliative . Others delay disease progression and the onset of complications associated with the underlying illness. Very few therapies in use today are capable of curing or significantly changing the course of disease. The result is a healthcare system burdened by costly treatments for an aging, increasingly ailing population, with few solutions for containing rising costs.*
- *The best way to significantly improve the economics of our current healthcare system is to develop more effective treatments for the most burdensome diseases and conditions—diabetes, neurodegenerative disorders, stroke and cardiovascular disease, for example—to facilitate longer, healthier and more productive lives.*
- *Regenerative medicine is uniquely capable of altering the fundamental mechanisms of disease; however, to realize its potential, we must think differently about therapeutic development and commit to investing in these transformative technologies.*

We deeply hope that these words focus and enlighten our discussions and make, as well, our debate useful for the scientific society and, ultimately, for patients.

Joan Garcia

ORGANIZING COMMITTEE



Joan Garcia, Director of XCELIA, Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia and Director of the Chair of Transfusion Medicine & Cellular and Tissue Therapies of the Autonomous University of Barcelona, Cerdanyola del Vallès, Spain

Scientific Leader of the event

Joan Garcia MD, PhD, Hematologist, with more than 30 years of experience on research and clinical application of cellular therapies, is the Director of XCELIA, the Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia (BST) (2009 to date). He is, as well, Associate Professor of the Autonomous University of Barcelona (2010 to date) and Director of its Chair of Transfusion Medicine & Cellular and Tissue Therapies (2012 to date). Former positions and commitments include: The Direction of the Barcelona Cord Blood Bank, one of the most important world wide (1994-2009), The Direction of the Department of Cellular Therapies of BST (2001 – 2009), The Direction of the Cryobiology and Cell Therapy Department of the Cancer Research Institute of Barcelona (1992-2001) and the Staff membership of the Department of Hematology of the Hospital de Sant Pau. (1982-1991). On 1999 he received the Honor Award of the Spanish Scientific Association. He has been co-founder and President of the International Netcord Association (2008-2010) and he has been author and co-author of a number of national and international papers and he has awarded by national and international research grants.



Sergio Querol, Director of the Cord Blood Bank and Cellular Therapies of the Banc de Sang i Teixits, Barcelona, Spain

Dr. Sergio Querol is currently Director of the Haematopoietic Progenitor Cell Unit and Cord Blood Bank of Banc Sang i Teixits in Barcelona (Spain). He also contributed in the establishment of the Anthony Nolan Cord Blood programme in UK where is now acting as Medical Consultant for Cord Blood Services. Dr. Querol was trained as physician in Valencia and after specialising in Haematology and Haemotherapy gained a Doctorate in ex vivo expansion of hematopoietic progenitors of umbilical cord blood for transplantation from the Universitat Autònoma de Barcelona. His publications cover the subject of cord blood and bone marrow transplantation: cord blood banking, stem cell expansion, characterization of cord blood cells, facilitation of haematopoietic engraftment and immunobiology of transplantation. Dr Sergio Querol's scope of interest is developing cellular therapy approaches in cord blood transplantation and developing a fast-track approach for accessing alternative donors in high risk leukaemia.



Joaquim Vives, R&D Director, XCELIA, Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia, Barcelona, Spain

Dr. Vives has 17 years' experience in the R&D biotech sector, focusing on the optimisation of cellular processes for high yield production of proteins from cells and the development of methods for obtaining high quality cells, either for drug screening or as a therapy. His current scientific interests at the Catalan Blood & Tissue Bank are related to the study of the unique properties of mesenchymal stromal cells as cell-based therapies in musculoskeletal pathologies. This involves the design, execution and analysis of GLP-compliant non-clinical studies using cells manufactured under GMP environments. Dr. Vives has a Master in Biotechnology and a PhD in Biochemistry from the Universitat Autònoma de Barcelona (Spain), and conducted post-doctoral research in Embryo Stem Cell Neurogenesis, at the Institute for Stem Cell Research (ISCR), University of Edinburgh (UK). Dr. Vives has also worked in the biotech sector, at Stem Cell Sciences Ltd (Cambridge, UK), where he focused on the development of novel applications of stem cells for the pharmaceutical industry. Author of 14 articles and 1 book chapter. Mentor of 4 PhD and 1 MRes theses.

Ruth Coll, Responsible of Clinical Development, XCELIA, Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia, Barcelona, Spain

Margarita Blanco, Responsible of GMP cell production, XCELIA, Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia, Barcelona, Spain

Margarita Codinach, Responsible of Quality Control, XCELIA, Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia, Barcelona, Spain

Elena Hernández, Director of Marketing and of the Banc de Sang i Teixits, Barcelona, Spain

Aurora Masip, Director of Communication of the Banc de Sang i Teixits, Barcelona, Spain

Laia Arnal, Head of Research and Scientific Debate, Biocat, Barcelona, Spain

INVITED SPEAKERS

Tuesday, February, 17th, 2015

KEYNOTE SPEAKERS

History of Cellular Therapies, Achievements, Challenges faced and milestones for the future



Alejandro Madrigal, Scientific Director of Research and Professor of Haematology of Anthony Nolan Research Institute, Royal Free and University College London, United Kingdom

Prof Madrigal is an internationally respected and influential scientist in the field of stem cell transplantation, and histocompatibility and immunogenetics, whose seminal research work has been the platform on which many recent advances in bone marrow transplantation have depended. His work has impacted upon clinical practice and has influenced the development of new tissue typing reagents by healthcare industries, and has defined viral and tumour specific targets that are used worldwide in vaccination and cell therapy. His achievements, as President of the European Group for Blood and Marrow Transplantation from 2010-2014, are recognised worldwide. Prof Madrigal began his medical career in Mexico and subsequently undertook a WHO Fellowship at Harvard University's

Dana Farber Cancer Institute; followed by a PhD degree at the Imperial Cancer Research Fund, London. He was awarded the Stanford Dean's Fellowship and a Leukemia Society Fellowship whilst a Postdoctoral Research Fellow at Stanford University. He has led research at Anthony Nolan since 1993, creating the Anthony Nolan Research Institute and leading as Scientific Director since 1995. He has held the Academic Chair of Haematology at the Royal Free Hospital and University College London since 1997. He has published over 570 papers, many in peer-reviewed journals (e.g., Nature, Lancet and PNAS) and has more than 5,330 citations. Prof Madrigal's work has been recognised worldwide and he has received several awards and prizes (such as the EBMT Stockton Prize and the ASHI International Scholarship Prize). He was awarded a Doctor of Science Degree in 2002 by UCL, has been honoured with two Doctor Honoris Causa degrees and many international awards, including in 2007, the Ohtli Award from the Government of Mexico and an Academic Distinction from the National Academy of Medicine in 2008. In August 2010, he received a Silver Award from the UK Department of Health, as a public acknowledgment of his expertise and the sustained and dedicated high quality of his work and contributions to the National Health Service. Prof Madrigal is a Fellow of the Royal Colleges of Pathologists and Physicians. He is International Advisor representing Mexico for the Royal College of Physicians. In October 2011 he was appointed as UCL Pro-Provost for the Americas and is currently Pro-Vice-Provost, and in May 2013 was elected as a Fellow of the prestigious Academy of Medical Sciences and in June 2014 as a Member of the Mexican Academy of Surgery.

Cellular Therapy - Present and Future Challenges

Therapeutic manipulation of the immune system may overcome morbidity and mortality caused by a deficient capacity for adaptive immunity and by malignancies not eradicable by other therapeutic interventions, surgery radiotherapy or chemotherapy. Interventions to modulate immune reactivity may be valuable for treatment of autoimmune and inflammatory chronic disease and manoeuvres to provide adaptive immunity, active or adoptive are of utmost importance to reduce the heavy burden of morbidity and mortality of patients and recipients. Different modalities of organs, tissues and cell transplants, and the efficiency of haematopoietic stem cell transplants to treat haematological malignancies may be enhanced by interventions directed to provide adoptive or to generate endogenous anti-tumour immune reactivity. Hence, if cell therapy is considered as a use of cells as therapeutic agents, two large areas can be differentiated, one involving regeneration, substitution or replacement of functional cells (stem cell therapy) and the other, related to the use of immune cells to exploit their specific types of responses in the effector and suppressor directions (cellular immunotherapy). Another potential application is regenerative medicine, in the context of degenerative diseases and/or autoimmunity. Large cord blood bank initiatives offer a unique opportunity to make available for research, a high number of ethically collected, clinical grade, cord blood units. Cells from cord blood can be grown and differentiated in various tissues including mesenchymal stem cells, bone, cartilage and other lineages. Future studies will test the potential of cord blood cells for the treatment of several diseases including, among other possibilities, diabetes, arthritis, burns, neurological disorders and myocardial infarction.



Sergio Querol, Director of the Haematopoietic Progenitor Cell Unit and Cord Blood, **Bank of Banc Sang i Teixits**, Barcelona, Spain

(See his CV at the Organizing Committee Section)

History of Cellular Therapies, Achievements, Challenges faced and milestones for the future

The hematopoietic stem cell transplantation (HPCT) was central in the development of cellularbased therapies. Key characteristic of the hematopoietic tissue, as a liquid organ, allowed individual cell analysis and facilitated understanding their physiological properties and consequently their clinical effects. HPCT introduced two effects on cancer therapy, the stem cell mediated tissue regeneration and the immune modulation derived from the adoptive innate and adaptive immunity. Cell therapy was further built on them: first, the regenerative ability led to the stem cell definition and its application in therapy on the broader field of regenerative medicine; on the other hand, alloreactivity phenomenon and the discrimination of graft versus host reactions contributed to the development of two key disciplines in transplantation, histocompatibility and cellular immunotherapy. Hematologists were pioneers in establishing first cryopreservation labs, which were the basis for later development of graft bioengineering facilities and finally the consolidation of cellular services as we know them today. Cellular therapy laboratories manufacture donor-derived pharmaceutical grade products after basic (minimal) manipulation or advanced technical production; the later when primary cells are ex vivo transformed by activation, expansion or gene manipulation. Main challenges for cell therapy products are how to confront though regulatory requirements similar to pharma industry that requires a well-defined product in research and, subsequently, designing and funding clinical trials to demonstrate safety and efficacy before routine use. Donor cell inter-variability and high complexity of biochemical pathways make difficult obtaining reproducible and universal products that can be easily scaled-up. Presentation will discuss current organization of HPC labs and main products currently used in clinical hematology.



Alejandro Madrigal, Scientific Director of Research and Professor of Haematology of **Anthony Nolan Research Institute, Royal Free and University College London**, United Kingdom

Co-chair of the **SESSION 1. TISSUE ENGINEERING AND REGENERATION. REAL CASES AND STANDPOINTS**

(See his CV at page number 11)



Anna Veiga, Director of the Cellular Lines Bank, **Center of Regenerative Medicine in Barcelona**, Barcelona, Spain

Co-chair of the **SESSION 1. TISSUE ENGINEERING AND REGENERATION. REAL CASES AND STANDPOINTS**



Julie Allickson, Director of the Regenerative Medicine Clinical Center, Wake Forest Institute for Regenerative Medicine, **Wake Forest University School of Medicine**, Winston-Salem, USA

Dr. Allickson focuses on the translation of regenerative medicine products including cell therapy, tissue engineering, biomaterials and devices. This process begins at Proof-of-Concept where early discussion with regulators and clinicians are critical in moving the technology from the bench to the bedside. The Translational Team includes Quality Assurance, Quality Control, Regulatory Affairs, Process Development and the GMP-complaint Manufacturing Facility. Prior to joining the institute,

she was an Executive Officer and Vice President of Laboratory Operations and R & D at Cryo-Cell International, Inc.

Dr. Allickson was part of the team to perform the very first Bone Marrow Transplant at the University of Miami in 1990 and has 25 years of experience in Cellular Therapy, Cellular Processing, Clinical Translation and Regenerative Medicine. She has a Doctorate in Health Sciences along with a Master's Degree in Medical Laboratory Sciences. She is one of the founding members of the International Society of Cellular Therapy in 1992 and has been a member of the American Association of Blood Banks (AABB) for 25 years. She has presented at national and international meeting related to adult stem cells and translation. She is currently Chair of the AABB Standards Committee for Cell Therapy Product Services. Dr. Allickson is also on the Technical Advisory Board for Tissue Engineered Products under ICCBBA and the ISCT Commercialization Committee.

Regenerative Medicine Therapies in the 21st Century

Regenerative Medicine represents a new paradigm in the field of healthcare with the potential technology for unmet clinical needs. As we are an aging population there is an increase in disease where regenerative medicine can play a significant role in the future. Currently there are more than 100,000 patients that need life-saving organs and only about 10,000 organ donors per year so tissue engineering could help to solve the organ donor shortage problem. Cells are a very important component of the field not only for direct therapy but also to build organs and tissues. As we assess replacement organs, the field weighs the pros and cons of decellularization of an organ compared to 3-D Bioprinting with the goal identifying an inexhaustible source of organs that will not require immunosuppression nor be rejected. This presentation will discuss the state of the field of Regenerative Medicine touching on Cell Therapy, Tissue Engineering, Bioprinting Technology, Translational Research Pathway and Regulatory Considerations as we transfer technology to the clinic. Understanding of the appropriate regulations coupled to early discussions with regulators can accelerate therapies to the clinic. Regenerative Medicine requires a multidisciplinary approach where many field join forces such as the combinations of scaffolds with cells, scaffold production, and new paradigms of the use of stem cells for tissue regeneration. Examples of the translational technology in regenerative medicine will be discussed.



José Luis Jorcano, Head of Division at **Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT)**, Madrid, Spain

José Luis Jorcano, PhD in Physics for the Complutense University of Madrid (1976, cum laude), has been the Chairman of the Epithelial Biomedicine Unit of CIEMAT (1987 to 2011), and has been the Managing Director of the Genome Spain Foundation (since its inception in 200 to 2009). At present he is Professor of Bioengineering at the Carlos III Institute in Madrid (UC3M) and the Director of the Joint Unit Ciemat-UC3M of Biomedical Engineering. It is Distinguished Fellow and Awarded by the Jovellanos-Havana Institute (in 2011). Silver Medal of Gijón City Hall Awards (in 2012) for his work on Molecular and Cellular Biology. He has developed his scientific career (1976-1987) in

prestigious European centers of biomedical research: Max-Planck Institut of Molecular Genetics (Berlin), Max-Planck Institute of Biology (Tübingen) and the German Cancer Research Center (Heidelberg). With 35 years of experience in Molecular and Cellular Biology, and more recently in Tissue Engineering and Gene Therapy. Elected member of the EMBO (European Molecular Biology Organization). It was Pfizer Ltd. and MD Anderson Cancer Research Center, Science Park, University of Texas Scientific Advisor. He is a corresponding member Royal Academy of Sciences of Spain.



Jaroslav Michalek, President of **International Consortium for Cell Therapy and Immunotherapy** and Professor in Pediatrics at **Masaryk University**, Brno, Czech Republic

Jaroslav Michalek, has completed his PhD in Pediatric Oncology at the age of 28 years at the Masaryk University in Brno, Czech Republic. Then he spent 3 years of postdoctoral studies related to cell therapies at the University of Texas Southwestern Medical Center at Dallas, TX, U.S.A. Recently he is president of the International Consortium for Cell Therapy and Immunotherapy (ICCTI) and CEO of Cellthera company (www.cellthera.cz) focusing on cell therapies. He is also the author of more than 130 scientific papers in reputed journals.

Treatment of Chronic Arthritis with Adipose Tissue Stromal Vascular Fraction

Therapy of osteoarthritis relies on non-steroid analgesics, chondroprotectives and in late stages total joint replacement is considered a standard of care. We performed a pilot study using novel stem cell therapy approach that was performed during one surgical procedure. It relies on abdominal lipoaspiration and processing of connective tissue to stromal vascular fraction (SVF) cells that typically contain relatively large amounts of mesenchymal stromal and stem cells. SVF cells are injected immediately to the target joint or to the connective tissue of the target joint. Since 2011, total of 1128 patients have been recruited and followed for up to 42 months to demonstrate the therapeutical potential of freshly isolated SVF cells. At the same time, one to four joints (knees and hips) were injected with SVF cells per patient. A total number of 1856 joints were treated. Clinical scale evaluation including pain, non-steroid analgesic usage, limping, extent of joint movement and stiffness was used as measurement of the clinical effect. All patients were diagnosed with stage II-IV osteoarthritis using clinical examination and X-ray, in some cases MRI was also performed to monitor the changes before and after stem cell therapy. After 12 months from SVF therapy, at least 50% clinical improvement was recognized in 91%, and at least 75% clinical improvement in 63% of patients, respectively. Within 1-2 weeks from SVF therapy 72% of patients were off the non-steroid analgesics and most of them remain such for at least 12 months. No serious side effects, infection or cancer was associated with SVF cell therapy. In conclusion, here we report a novel and promising therapeutical approach that is safe, cost effective, and relying only on autologous cells.



Massimo Dominici, President of the **International Society for Cellular Therapy (ISCT)** and Assistant Professor in Oncology and Hematology at the **University of Modena and Reggio Emilia**, Modena, Italy

Dr. Massimo Dominici started his brilliant career with an MD degree at the University of Pavia (Italy) in 1996. His medical residency was in Haematology in the Ferrara University (Italy) between 1996 and 2000. In 2000 he started being Postdoctoral research associate in the St. Jude Children's Hospital, Memphis (USA) until 2003. He worked as hospital physician, being assistant professor of Medical Oncology and head of the Laboratory of Cellular Therapies at University/Hospital of Modena where he coordinates 12 Collaborators. In December, 2013 became associate professor of Medical Oncology (Nat. Board on Scientific Habilitation). He has received twenty three between research grants and awards. He has published eighty-four papers on stem cells, tissue regeneration, experimental oncology and hematology with over 8000 citations. He is author of 3 books, 3 chapters. He also has six deposited patents. Massimo Dominici is the founder of the University start-up Rigenarand, founder and Scientific Coordinator of the Mirandola Science & Technology Park. Co-Editor of Cytotherapy, member of the Editorial Board in the Word Journal of Stem Cells, the American Journal of Cancer Research, the Niche, the Frontiers in Stem Cell Treatments and of the Oncology & Hematology Review. He is reviewer for more than 40 scientific Journals and for 13 national & international founding Bodies. He is founder of the Forum of Italian Researcher on MSC. In 2012 hi was Scientific Advisor for the Italian Minister of Health on stem cells. He has been member of ISCT, ASH, ESCGT, IFATS and board member of JAICE. He is the current President of the International Society for Cellular Therapy (ISCT).

Position statement of the international society for cellular therapy (ISCT)



Agustin Zapata, Professor of Cell Biology at **Complutense University of Madrid**, Madrid, Spain

Co-chair of the **SESSION 2. WHICH CELLS? FIVE SOURCES TO CHOOSE FROM**



Massimo Dominici, President of the **International Society for Cellular Therapy (ISCT)** and Assistant Professor in Oncology and Hematology at the **University of Modena and Reggio Emilia**, Modena, Italy

Co-chair of the **SESSION 2. WHICH CELLS? FIVE SOURCES TO CHOOSE FROM**

(See his CV at the page 14)



Joaquim Vives, R&D Director, XCELIA, Advanced Therapies Division of the Banc de Sang i Teixits of Catalonia, Barcelona, Spain

(See his CV at Scientific Committee Section)

Biological Properties and Clinical Grade Production Development of Bone Marrow Mesenchymal Stromal Cells

Mesenchymal Stromal Cells (MSC) have been proposed as drug candidates for treatment of a variety of pathological conditions and injuries. MSC were originally discovered as a rare subset of bone marrow-residing haematopoietic stem cells, and they can be isolated at present from multiple tissues, including fat and umbilical cord. Despite the extensive literature on MSC, substantial ambiguities still exist regarding their identity, potency, function, methods for isolation and ex vivo expansion, and mode of action in the clinical setting. Provided that prospective isolation is hindered by the lack of a unique marker, the International Society for Cellular Therapy have proposed three criteria to define MSC: (1) the plastic adherence of the isolated cells in culture, (2) the expression of CD105, CD73 and CD90 in greater than 95% of the culture, and their lack of expression of markers including CD34, CD45, CD14 or CD11b, CD79a or CD19 and HLA-DR in greater than 95% of the culture, and (3) the differentiation of the MSC into osteoblasts, adipocytes and chondroblasts in vitro. However, bulk MSC cultures fulfilling such criteria are actually composed of heterogeneous cell populations, which is well illustrated by the differential growth and developmental potentials exhibited by individually expanded MSC clones. Therefore data obtained from different laboratories using proprietary methods for MSC production may not be comparable. Furthermore, the discovery of paracrine properties of MSC has increased the range of therapeutic applications as trophic, anti-inflammatory or immune-modulator agents, instead of as progenitor cells, making more difficult to understand the cellular mechanisms involved, especially in the context of allogeneic cell therapy, in which the cells are cleared within hours after administration. All these uncertainties may have a direct impact on their envisioned therapeutic exploitation.



Damián García Olmo, Professor of Surgery and Chief of Surgery Department at Fundacion Jimenez Díaz University Hospital, **Universidad Autónoma de Madrid**, Spain

Dr. Damián Garcia-Olmo is currently Professor of Surgery and Chief of Surgery Department at Fundacion Jimenez Díaz University Hospital (Universidad Autónoma de Madrid, Spain). He's more than 25 years of experience both in the clinical setting as well as in research. His Team has been world pioneer in the use of adult mesenchymal stem cells derived from adipose tissue (ASC) in human cell therapy. From 2002, they led a clinical trial process about the use of ASC for the treatment of Crohn's disease including Phase III clinical trials. As relevant events can be noted as follow: five international patents in the field of Regenerative Medicine, two cells medicines developed and Two companies created based to this scientific activity. In the last 15 years, his Group has been also investigating, in collaboration with other international research groups, in analyzing the transforming ability of Circulating Nucleic Acids in Plasma/Serum (CNAPS) from patients with colorectal cancer on ASC. This

was previously demonstrated in vitro and in animal models, suggesting an infectious origin of cancer, and was tentatively named as the Genometastasis Theory (original theory).

Adipose Tissue Mesenchymal Stromal Cells Properties and Clinical Application

Mesenchymal stem cell (MSC) research has developed rapidly during the last decade and the promising results obtained from in vitro and in vivo studies have generated growing optimism. Although bone marrow is the most often used source, MSCs with similar biological properties have also been isolated from other tissues including adipose tissue, skeletal muscle and cord blood. Of special interest is adipose tissue since it represents an abundant, safety and accessible source of MSCs. These cells are denominated adipose-derived stem cells (ASCs) and have been widely studied since they were first described in 2001. In recent years, substantial knowledge of ASCs interaction with the immune system has been acquired. The mechanisms underlying the immunosuppressive effects of ASCs have not been clearly defined but it seems that ASCs modulate the function of different cells involved in the immune response. In this way, it is evident their anti-inflammatory activity in several experiments. Recent studies address the potential benefit of application of MSCs in systemic acute inflammatory response in septic shock. In this scenario, ASCs with their potential and encouraging properties could be helpful. In 2014, the most advanced programmes, involving Crohn's disease and fistulous disease, have reached phase III of development. Other tested diseases include, GVHD, Rheumatoid Arthritis or Myocardial Infarction. If the preliminary results are confirmed, we think that cells therapy using ASCs as a cell source, may become in a clinical reality in the near future.



Alan Smith, Vice President of Research and Development and Cellular Therapeutics for **LifeNet Health**, Virginia Beach, USA

Alan Smith, PhD is well-seasoned executive and experienced researcher with a background of over 30 years in the Research and Development industry. He is presently the Vice President of Research & Development and Cellular Therapeutics for LifeNet Health and its wholly owned subsidiary, The Institute of Regenerative Medicine. Here he is responsible for research, product development, cell production, research tissue, and cardiovascular research for the company including overall program management and general operations of the Institute. Prior to this position, Dr. Smith was an independent consultant after serving as President and Chief Executive Officer for Cognate BioServices for eight years. He has held senior management positions with Osiris Therapeutics, Aastrom Biosciences, Genetic Sciences, Baxter Healthcare Corporation, and others. Dr. Smith is a published co-author on numerous journal articles and is an Adjunct Professor at Eastern Virginia Medical School, Department of Biology and Molecular Cell Biology. His past academic experience includes adjunct professorships at California State University Long Beach and Utah, State University. Dr. Smith earned his BS degree in Chemistry at Southern Utah University in 1976 and PhD degree in Biochemistry from Utah State University in 1982. He currently serves on the Board of Directors for CHD Bioscience, Inc., MGP Technologies, Inc., and for American Qualex, Inc. where he also serves as Chairman of the Scientific Advisory Board.

Cellular Bone Matrix, a Differentiated Cellular Allograft for the Repair or Reconstruction of Musculoskeletal Defects

An ideal bone allograft material should contain osteogenic, osteoinductive and osteoconductive elements. The success of standard acellular bone allografts in patient treatment depends on the availability and coordinated participation of the patient mesenchymal stem cells (MSCs) to deposit bone in conjunction with the inherent scaffold and bone-promoting growth factors found in the allograft bone. However, MSCs are limited in number with their incidence reduced with age. ViviGen Cellular Bone Matrix, developed by LifeNet Health, is comprised of cryopreserved live, viable bone cells within a corticocancellous bone matrix with demineralized bone added. The bone cell populations found in ViviGen were characterized for osteogenic potential and rate of bone formation compared to that of mesenchymal stem cells. ViviGen derived cells express higher levels of osteopontin, osteocalcin, and bone morphogenetic protein-2 than MSCs. Osteogenic cells in ViviGen exist in a committed and primed state in an environment conducive to bone formation. These cells deposit calcium at a significantly faster rate than mesenchymal stem cells in vitro. Additionally, the bone-forming cells show a lack of immunogenicity. ViviGen represents a viable alternative to autograft and acellular allograft in bone grafting procedures.



Hermann Einsele, Director of the Department of Internal Medicine II, and Full Professor and Chair for Internal Medicine II, **University of Würzburg**, Germany

Prof. Einsele is considered to be one of the world's leading experts in the field of multiple myeloma, stem cell transplantation and invasive aspergillosis in haematological and oncological patients and has published over 400 articles in peer-reviewed journals. His research interests include multiple myeloma, stem cell transplantation, adoptive immunotherapy as well as viral and fungal infections in immunocompromised host.

T Cell Immunotherapy for Infectious Diseases

Viral infections are still common causes of morbidity and mortality in immunosuppressed patients after allogeneic hematopoietic stem cell transplantation. Infections caused by virus such as cytomegalovirus, adenovirus and Epstein-Barr virus are well-known. In addition, several other viruses have been recently reported to be causes of significant complications. As the delay in recovery of virus-specific cellular immune response after transplant is associated with viral reactivation and viral disease, adoptive immunotherapy to restore virus-specific cellular immunity is an attractive option. Recent clinical trials showed the safety and effectiveness of adoptive immunotherapy against viral diseases. This presentation will summarize the current status of adoptive immunotherapy against several viral diseases including cytomegalovirus, adenovirus, Epstein-Barr virus and fungal infections.



Ángel Raya, ICREA Research Professor at **Institute for bioengineering of Catalonia (IBEC)** and Director at **Center of Regenerative Medicine in Barcelona (CMRB)**, Barcelona, Spain

Ángel Raya holds an MD and a PhD from the University of Valencia. He pursued postdoctoral training at the Instituto de Investigaciones Citológicas (currently, Centro de Investigación Príncipe Felipe) in Valencia, from 1995 to 2000. He then was a Research Associate (2000-2004) and a Senior Research Associate (2004-2006) in the Gene Expression Laboratory of the Salk Institute for Biological Studies, La Jolla, CA (USA). He returned to Spain in 2006 as an ICREA Research Professor. He was Scientific Coordinator at the Center of Regenerative Medicine in Barcelona (CMRB) until 2009, when he joined the Institute for Bioengineering of Catalonia (IBEC) as group leader of the Control of Stem Cell Potency Group. In 2014 he was appointed Director at CMRB.

Induced Pluripotent Stem (iPS) Cells for Disease Modeling and Treatment

Takahashi and Yamanaka were the first to generate induced pluripotent stem (iPS) cells by nuclear reprogramming of other cell types with defined combinations of transcription factors. Ever since, a large number of laboratories worldwide have validated the technique, and in fact iPS cells can now be produced routinely from multiple species (including humans) and using multiple methods. The implications of reprogramming in general and of human iPS cells in particular are vast. On one hand, human iPS cells are providing outstanding models for drug/toxicity screening and disease mechanistic studies. In this respect, I will summarize the efforts from our lab and others on the use of patient-specific iPS cells to model neurodegenerative diseases. On the other hand, iPS cell technology also holds promise for future cell-based therapies; here, I will discuss current limitations of this strategy and present ongoing approaches to obtain mature, functional cardiomyocytes for cell replacement therapy of heart failure.



Francisco Maculé, Head of the Knee Unit at **Hospital Clínic**, Barcelona, Spain

Co-chair of the **SESSION 3. FOR WHAT DISEASES? WHO CAN BENEFIT FROM CELLULAR THERAPY NOW AND IN THE NEAR FUTURE?**



Damián García Olmo, Professor of Surgery and Chief of Surgery Department at **Fundación Jimenez Díaz University Hospital, Universidad Autónoma de Madrid**, Spain

Co-chair of the **SESSION 3. FOR WHAT DISEASES? WHO CAN BENEFIT FROM CELLULAR THERAPY NOW AND IN THE NEAR FUTURE?**

(See his CV at page 15)



Manuel Galiñanes, Head of the Department of Cardiac Surgery and of the Reparative Therapy of the Heart at the **University Hospital Vall d'Hebron, University Autònoma of Barcelona**, Barcelona, Spain

Dr. Galiñanes has more than 30 years of clinical and research experience. He worked as a cardiac surgeon and senior investigator from 1987 to 2009 in two United Kingdom University Hospitals; first at the University Hospitals of Guy' and St Thomas' NHS Trust (London) and later at the University Hospitals of Leicester NHS Trust, Leicester. At present, he is the Head of the Department of Cardiac Surgery and of the Reparative Therapy of the Heart at the University Hospital Vall d'Hebron, University Autònoma of Barcelona, Spain. Dr Galiñanes research interests are focused in the molecular basics of cardioprotective treatments and reparative therapies of the heart. His group is

linked to several other groups, nationally and internationally, to collaborate in basic and clinical studies with an interest on translational research.

Heart Diseases: Who can benefit from cellular therapy now and in the near future?

It is now generally accepted that the mammalian heart possesses resident stem cells and the capacity to repair itself. The availability of various stem cells for clinical use has made cell therapy a promising tool to repair different cardiac conditions, including acute myocardial infarction, heart failure, refractory angina caused by chronic ischemic heart disease and malfunction of the excitation and conduction system. Despite encouraging results in animal experimental models, the clinical trials have not provided the expected benefit. The cause for the reported inconsistent results in clinical trials in which cell therapy has been used may be due to the selection of patients to receive the treatment, and to differences in the cell types applied and their mode of application.



Bernat Soria, Director of the **Stem Cells Department in CABIMER (The Andalusian Center of Molecular biology and Regenerative Medicine)**, Seville, Spain

Bernat Soria is Director of the Stem Cells Department of the Andalusian Center for Molecular Biology and Regenerative Medicine (CABIMER), Director of the Andalusian Cell Therapy and Regenerative Medicine Program and the Observatory for International R+D Alliances. He was Former Minister of Health and Consumer Affairs of Spain (2007-2009), and he was previously Professor of Physiology in Alicante (1986-2005). Between 2005 and 2007 he was Director of CABIMER (Andalusian Center for Molecular Biology and Regenerative Medicine in Seville, Spain) and Professor of Regenerative Medicine at the University Pablo de Olavide in Seville. He graduated from Valencia University (Spain) and completed his postdoctoral studies at the Max Plank Institute for Biophysical Chemistry (Göttingen, Germany) and at the University of East Anglia, School of Biological Sciences (UK). He has been Visiting Prof in the National University of Singapore, Chairman of the European Stem Cell Network and President of the European Association of Biophysical Societies. He has previously held posts such as the Presidency of the Spanish Society of Diabetes, the Spanish Society of Biophysics and the Spanish Society of Physiological Sciences. He has published more than 125 papers and edited 4 books. His work has received more than 5000 quotations in the fields of stem cell research, pancreatic islet biophysics and pathophysiology. His publication in *Diabetes* (2000) pioneered the field of stem cell differentiation into insulin producing cells. The use of stem cells in the treatment of diabetes and their complications focus his current interest. Several of his former students are current Professors in the areas of Physiology, Biophysics, Nutrition, Applied Biology, etc. Among others he has received the Prize and Gold Medal of the Royal Academy of Medicine, the Galien International Prize, the Medal of Andalucía and, more recently, the High Cross of the Carlos III Order from the King of Spain.

Diabetes Mellitus. How Can Be Treated by Cellular Medicines?



Antonio Uccelli, Director **Centre of Excellence for the Biomedical Research (CEBR) - University of Genoa**, Genoa, Italy

Antonio Uccelli obtained his medical doctoral degree in 1987, in the University of Genoa. He completed his residency of neurology at the University of Genoa in 1993. In 1992 he attended, as post-doctoral fellow, the Laboratory of Neuroimmunology - Department of Neurology - University of California San Francisco (UCSF) directed by Professor S.L. Hauser. From 1993 to 2011 he had a faculty position in the Department of Neurology of the University of Genoa. Since February 2012 he is the Coordinator of the Residency School of Neurosurgery of the University of Genoa. Since December 2011 he is Associate Professor of Neurology of the University of Genoa. Since 2014 he is Director of Centre of Excellence for Biomedical Research (CEBR). Since 1995 he is the Director of the Neuroimmunology Unit of his Department focusing his research activities on multiple sclerosis and more recently on adult stem cells. In 2001 he received the Rita Levi Montalcini Award, yearly assigned to the best Italian researcher in the field of MS. In 2009 received the Melvin Jones Fellow Award (for dedicated humanitarian services LIONS CLUBS INTERNATIONAL FOUNDATION) and in 2013 the Royan International Research Award - an annual prize awarded to the excellences in Reproductive Biomedicine, Reproductive Health, and Stem Cell Biology and Technology. Since 2001 he joined the Center of Excellence for Biomedical Research of the University of Genoa of whom it became the Director since March 2014. Since 2008 he is the Director of the Neuroimmunobiology Laboratory of the Advanced Biotechnology Center of Genoa. Since 2009 he is the Director of the Center for Research and Cure of Multiple Sclerosis of the University of Genoa. Prof. Uccelli is co-author of 126 peer-reviewed scientific publications. Prof. Uccelli's C.I. (Citation Index) is 9322 and H Index is 42. He has been invited to give seminar and keynote lectures at many Academic sites and conferences all over the world. Since 1995 he has received numerous scientific grants from national and international agencies.

Mesenchymal Stem Cells for Multiple Sclerosis: Hypes and Hopes

Recent advances in our understanding of stem cell biology, such as the availability of innovative techniques, which allow stem cells to be obtained on a large scale, and the increasing pressure from patients for tissue repair strategies, have launched stem cell treatments as one of the most exciting and difficult challenges in the multiple sclerosis (MS) field. The adult stem/progenitor cells from bone marrow and other tissues referred to as mesenchymal stem cells or multipotent mesenchymal stromal cells (MSC) display a significant therapeutic plasticity as reflected by their ability to enhance tissue repair and influence the immune response both in vitro and in vivo. Pivotal experiments have been carried out in experimental autoimmune encephalomyelitis (EAE), a model for MS demonstrating that intravenous MSC administration induces tolerance to myelin antigens and promote as well neuroprotection and endogenous neurogenesis without evidence of significant engraftment or transdifferentiation. Thus, current experimental evidence suggests that the sound clinical exploitation of stem cells for MS may lead to novel strategies aimed at blocking uncontrolled inflammation, resetting the immune system, protecting neurons and promoting remyelination, but not at restoring the chronically deranged neural network responsible for irreversible disability typical of the late phase of MS. Based on these results, several small pilot clinical trials in subjects with advanced MS have demonstrated that MSC administration is safe and provided an early signal of clinical effectiveness. The current aim of clinicians and scientists interested in the development of MSC-based strategies for the treatment of MS is to have the ultimate demonstration in large clinical trials that MSC can inhibit CNS inflammation and foster tissue repair as realized clinically, with functional recovery, or visualized by magnetic resonance imaging (MRI). A large phase II multicenter clinical trial (MESEMS) to answer these questions is currently on going and the results are expected by early 2016.



Enric Càceres, Vice President of the **European Federation of National Associations of Orthopaedics and Traumatology (EFORT)** and Chair of Orthopedics and Traumatology Surgery of **Universitat Autònoma de Barcelona**, Cerdanyola del Vallès, Spain

Enric Càceres is an international prestigious specialist in Traumatology and Orthopaedics. He is Chair of Orthopedics and Traumatology Surgery of Universitat Autònoma de Barcelona since 2009, and he became Vice President of the European Federation of National Associations of Orthopaedics and Traumatology (EFORT) in 2013. He is an Associate Professor of the Health Policy&Management Department of the Johns Hopkins University, Baltimore, Maryland, USA. He works as a Medical Advisor of Football Club Barcelona and he is member of the World Motorbike Championship's Medical Team since 2012. He is the Head of the Spine Unit of the Instituto Universitario Quirón/Dexeus, in Barcelona and he is also the Head of the Orthopaedics Department of Vall d'Hebron Hospital. In December 2014 he was recognized for MERCO as the second best Orthopaedics and Traumatology surgeon of Spain.

Bone and Cartilage Regeneration with Cells and Tissue Engineering Products

Stem cells and progenitors that are capable of forming new tissue with one or more connective tissue phenotypes are available from many adult tissues and are defined as connective tissue progenitors. There are four cell-based tissue-engineering strategies: A. targeting local connective tissue progenitors where new tissue is desired; B. Transplanting autogenous connective tissue progenitors, C. transplanting culture-expanded or modified connective tissue progenitors and D. transplanting fully formed tissue generated in vitro or in vivo. See comment in PubMed Commons below. We could expose the experience whit Barcelona– BST stem cells trials in orthopaedics: 1) Multicentre study using stems cells culture in the treatment by fusion of degenerative spondylolisthesis in adults, 2) Treatment of knee osteoarthritis with aoutologous mesenchymal stem cells, 3) Intervertebral disc repair by autologous mesenchymal bone marrow cells, 4) Use of autologous mesenchymal bone marrow cells in meniscal injuries repair.



Javier Briones, Chief of Section of Hematology at **Hospital Santa Creu i Sant Pau**, Barcelona, Spain

Dr. Javier Briones is Chief of Section of Hematology at Hospital Santa Creu i Sant Pau and associate professor of Medicine at Universidad Autónoma de Barcelona. He was an oncology fellow at Stanford University (USA) in Ron Levy's lab, where he did research studies on immunotherapy and gene therapy of lymphoma.

Cellular Immunotherapies for Hematological Malignancies

Cancer immunotherapy has been the subject of intensive research in the last decade. A better understanding of the biology of cells involved in the immune response together with the knowledge of how tolerance and tumor immunosuppression regulates antitumor immune response have contributed to the development of improved cancer immunotherapies. These include the development of chimeric-antigen receptor (CAR) redirected T cells and novel NKT cells agonists. In addition, targeting immunoregulatory cells and T cell inhibitory molecules with monoclonal antibodies are becoming a relevant approach for the treatment of cancer.

INVITED SPEAKERS

Wednesday, February, 18th, 2015

Elena Hernández, Director of Marketing and of the Banc de Sang i Teixits, Barcelona, Spain

Aurora Masip, Director of Communication of the Banc de Sang i Teixits, Barcelona, Spain

Summary of the sessions held the day before

Lluís Puig, Director of the Blood Division of the Banc de Sang i Teixits, Barcelona, Spain

Blood Tissue services and products:

- Blood Components
- Transfusion service
- Umbilical Cord Bank
- Tissue Bank
- Human milk Bank
- XCELIA. Advanced Therapies
- Biobanc

Visit to the blood and tissue bank facilities





José María Moraleda, Director of Spanish Cell Therapy Network (TERCEL) and Professor of Hematology at University of Murcia, Hospital Clínico Universidad Virgen de la Arrixaca, Murcia, Spain

Chair of the **OPEN SESSION: FROM BENCH TO THE BEDSIDE**



Marcos Timón, Head of Service at the Unit for Advanced Therapies and Biotechnology, Spanish Medicines Agency (AEMPS), Madrid, Spain

Marcos has more than 17 years experience in basic research in the field of Immunology, developed in Spain and the UK. He was Scientific Director at Mologen, a German biotech company, for 5 years. Since joining AEMPS, he has been mostly involved in the assessment of medicinal products of biological origin. He is the alternate member for Spain to the Committee for Advanced Therapies (CAT) at the EMA in London, and a member of several expert groups at AEMPS and at the European Directorate for the Quality of Medicines (EDQM) in Strasbourg (France).

Advanced Therapies Medicinal Products (ATMPs) Regulations in the EU and Spain

Advanced therapy medicinal products (ATMPs) comprise a diverse group of therapeutic approaches classified into three main classes: gene therapy, cell therapy and tissue engineered products. Regulation 1394/2007 created a specific and harmonised regulatory framework for ATMPs in Europe, aimed at guiding the translation of these innovative treatments from the bench to the clinic and, eventually, to make them widely available for patients via a marketing authorization, just as any other medicinal product. Fulfilment of the quality, safety and efficacy requirements for this type of products pose many challenges for both developers and regulators. This presentation will discuss the most important aspects of the ATMP regulation in Europe and the experience gained during the 6 years that have passed since coming into force. Implementation of the so called “hospital exception” in Spain will also be presented.



Nina Bauer, Business Development Executive EU, Cell Therapy Catapult, London, UK

Nina has over 15 years’ experience in the life sciences sector, working in a number of business development roles in the UK. Her main focus is on engaging both academic and commercial entities and their respective expertise, with the aim of developing an end-to-end cell therapy industry in the UK. At the heart of this work lie five core pillars: (1) helping the industry deliver life changing cell therapies to patients; (2) growing and sharing experience and expertise to lead the transformation of the cell therapy industry; (3) collaboration to increase efficiency and produce the best possible outcomes to help drive growth; (4) managing and lowering risk for long-term investment; and (5) making the UK the most compelling and logical choice for international partners. Nina has a Master in biology from Stony Brook University (NY, USA), a PhD in neuroscience from the University of Oldenburg (Germany), and conducted post-doctoral research in neuroscience and stem cell research at the Weizmann Institute (Israel), the Salk Institute (CA, USA), and at the University of Edinburgh (UK).

Relevance of Pre-Clinical Development. The Catapult Experience

The development of a cell based therapies requires a case-by-case approach to nonclinical development and safety. The programme depends on many factors including the type of cell therapy, the differentiation status and proliferation capacity of the cells, the route of administration, the intended clinical location, long term survival of the product and/or engraftment, the need for repeated administration, the disease to be treated and the age of the population. Understanding the product profile of the intended therapy is also crucial to the development of the nonclinical development programme. The Cell Therapy Catapult established by Innovate UK in 2012 to help grow the UK cell therapy industry provides expert advice into the assessment of the non-clinical safety profile of candidate programmes. This involves the planning and implementation of nonclinical studies and safety studies.



Eduardo Bravo, Managing Director and Chief Executive Officer (CEO), **Tigenix**, Madrid, Spain

Mr. Eduardo Bravo has more than 25 years' experience in the biopharmaceutical industry. Prior to joining the company in 2005 he held several senior management positions at Sanofi-Aventis, including Vice President for Latin America, a division with 2000 employees and sales of more than EUR 1 billion. At Sanofi-Aventis he also held senior positions in Marketing and Sales for Europe and he was General Manager for Belgium. Prior to his tenure at Sanofi-Aventis, Mr. Bravo spent 7 years at SmithKline Beecham in commercial positions both nationally and internationally. Mr. Bravo holds a degree in Business Administration and an MBA (INSEAD). He is Vice-President of EBE (European Biopharmaceutical Enterprises) and member of the Executive Committee of ARM (Alliance for Regenerative Medicine).

The Tigenix Case: the First Company Having a EMA Registered ATM

TiGenix is a leading European cell therapy company with an advanced clinical stage pipeline of adult stem cell programs and one commercial product, ChondroCelect. TiGenix is based out of Leuven, Belgium, and has operations in Madrid, Spain. ChondroCelect, indicated for cartilage repair in the knee, is to date the only approved cell-based product in Europe. It is the first cell-based product that successfully completed the entire development track from research, through clinical development to European approval through the centralized procedure. ChondroCelect received Marketing Authorisation in October 2009, as the first Advanced Therapy Medicinal Product under the new regulation for Advanced Therapies and was approved for reimbursement in Belgium in February 2011, in the Netherlands in June 2012 (retroactively to January 2011) and in Spain in March 2013. Effective, 1 June 2014, the company has entered into a distribution agreement with Sobi (Swedish Orphan Biovitrum AB) for the exclusive marketing and distribution rights with respect to ChondroCelect in Europe (excluding Finland, where TiGenix has a pre-existing distribution agreement with Finnish Red Cross Blood Services), the Middle East and North Africa.

Concluding remarks



Joan Garcia, Director of XCELIA, Advanced Therapies Division of the **Banc de Sang i Teixits of Catalonia** and Director of the Chair of Transfusion Medicine & Cellular and Tissue Therapies of the **Autonomous University of Barcelona**, Spain



José María Moraleda, Director of **Spanish Cell Therapy Network (TERCEL)** and Professor of Hematology at **University of Murcia, Hospital Clínico Universidad Virgen de la Arrixaca**, Murcia, Spain

PRACTICAL INFORMATION

Venues



Tuesday, February, 17th, 2015

CosmoCaixa Barcelona

Agora Room (-3 floor)

C/ Isaac Newton, 26 08022 Barcelona, Spain

obrasocial.lacaixa.es/laCaixaFoundation/home_en.html



Wednesday, February, 18th, 2015

Banc de Sang i Teixits Barcelona

Edifici Dr. Frederic Duran i Jordà

Passeig Taulat, 116 08005 Barcelona, Spain

www.bancsang.net

Speaker's hotel



Hotel ABBA Balmoral Barcelona

Via Augusta, 5

08006 Barcelona, Spain

Phone: +34 932178700

www.hotelbalmoral.com

Contact person during the event



Laia Arnal

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OUTCOMES

B·Debateca

On the website of **B·Debate**, you will find all the information related with the celebration of the meeting that includes reports, conclusions, scientific documents, interviews with the experts, speaker's CVs, videos, images, press documentation and other related materials. We invite you to visit the section **B·Debateca** on www.bdebate.org

Contents of the meeting **"ADVANCED CELLULAR THERAPIES AND REGENERATIVE MEDICINE. THE PROMISE IN THE 21ST CENTURY"**

The screenshot shows the B-DEBATE website interface. At the top, there is a navigation bar with tabs: HIGHLIGHTS, B-DEBATE, CALL FOR PROPOSALS, B-DEBATECA (circled in red), NEWS, and CONTACT. Below the navigation bar, there are filters for DEBATE (Cellular Therapies, circled in red), CONTENTS (All), TOPICS (All), and DATE (All). The main content area displays a list of speakers for the event "Advances Therapies and Regenerative Medicine. The Promise in the 21st Century" (dated 17/02/2015). The speakers listed are:

- Hermann Einsele (Würzburg University)
- Joan Garcia (Banc de Sant i Teixits (BST))
- Joaquim Vives (Banc de Sang i Teixits (BST))
- Sergi Querol (Barcelona Cord Blood Center)
- Marcos Timón (Agencia Española del Medicamento y Productos Sanitarios (AEMPS))
- Alejandro Madrigal (Anthony Nolan Trust, London (UK))
- Julie Allickson (Wake Forest Institute (USA))

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B-Debate International Center for Scientific Debate Barcelona is a **Biocat** initiative with support from “**la Caixa**” **Foundation**. It drives first-rate international scientific debates, to foster dialogue, collaboration and open exchange of knowledge with prestigious national and international experts, to approach complex challenges of high social interest in life sciences. B-Debate sees debate as a powerful, effective way to generate knowledge and strives to help position Barcelona as a benchmark in generating knowledge and Catalonia as a country of scientific excellence.

B-Debate sees debate as a powerful, effective way to generate new knowledge. The debates are top-notch international scientific meetings featuring a selection of experts of renowned international prestige and scientists that work in Barcelona and Catalonia, moderated by scientific leaders. Since 2009 B-Debate has invited about 800 recognized speakers and over 4.000 attendees. B-Debate seeks out answers to the challenges and needs of society in the field of life sciences, taking into account the complex, ever-changing conditions of this global world. The debates foster the integration of different disciplines of science and deal with such diverse topics as ageing, new therapeutic approaches to various diseases, innovative technology to improve knowledge of the human genome, food resources, new tools to integrate knowledge management, clinical genomics, neurosciences, climate change, and new energy sources, among others. The knowledge and results obtained through these events is spread throughout both the scientific community and general society through the various **B-Debate** channels and instruments.

More info: www.bdebate.org



The **Banc de Sang i Teixits** (Blood and Tissue Bank) is the public company of the Health Department that manages and administrates the donation, analysis and transfusion of blood and blood plasma. We also act as a centre for tissue collection and processing as well as developing other lines of activity in the field of immunobiology, molecular diagnosis, cell therapy and regenerative medicine. Its **Advanced Therapies Division, XCELIA**, develops and clinically tests personalized medicines based on cellular therapies and tissue engineering.

More info: www.bancsang.net

COLLABORATORS

CosmoCaixa ^{Barcelona}

CosmoCaixa offers interactive, enjoyable science and an open door for anyone who is eager to learn and understand and who never stops wondering why things are the way they are. **CosmoCaixa Barcelona** boasts the Geological Wall and the Amazon Flooded Forest, which features more than 100 plant and animal species that convince visitors they have been transported from the Mediterranean to the very heart of the tropical jungle. In addition to its permanent facilities and its open areas, CosmoCaixa offers a scientific and educational programme that includes exhibitions, workshops, conferences, courses and debates involving experts from all over the world.

More info: obrasocial.lacaixa.es



TerCel is a network of researchers working in Spain in order to improve, expand and disseminate knowledge about stem cells and the potential that they have, for clinical use. TerCel network believe that the future of medicine lies in the development of this potential to transfer to clinical practice these new medical therapies.

More info: www.red-tercel.com



The **Transfusion Medicine and Advanced Cell Therapies Chair (CMT3)** mission is to promote research, education and training on Transfusion Medicine and Cellular and Tissue Therapies among students and professionals of health and life sciences field, in order to improve their skills and professional development. It was created in 2008 by agreement between The Universitat Autònoma de Barcelona (UAB), the Banc de Sang i de Teixits of Catalonia (BST), and the Fundació Doctor Robert (FDR). The CMT3 develops a number of educational activities including an International Master on Transfusion Medicine and Advanced Therapies.

More info: cmt3.cat

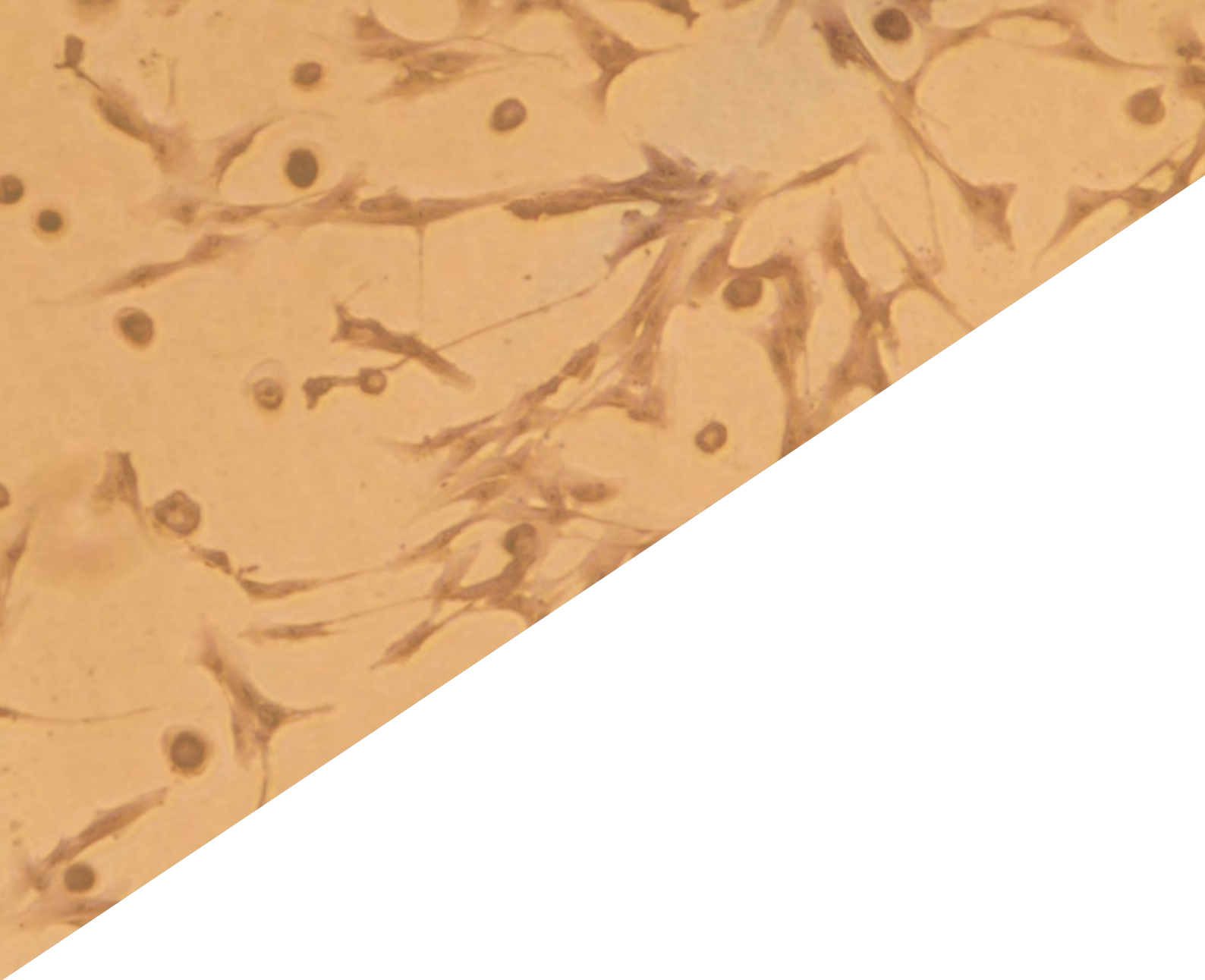


ISCT is a global society of clinicians, regulators, technologists, and industry partners with a shared vision to translate cellular therapy into safe and effective therapies to improve patients' lives. ISCT Members gain access to an influential global community of peers, experts, and organizations invested in cell therapy. ISCT Mission is "To drive the translation of all cellular therapies for the benefit of patients worldwide".

More info: www.celltherapysociety.org

NOTES

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VENUE TUESDAY FEBRUARY, 17TH:



**COSMOCAIXA
BARCELONA**
C/ Isaac Newton, 26
08022 Barcelona

VENUE WEDNESDAY FEBRUARY, 18TH:



**BANC DE SANG
I TEIXITS**
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