

ADVANCED THERAPIES

SCIENTIFIC BASIS
AND CLINICAL USES

Directors:

Prof. José María Moraleda Jiménez

Prof. Robert Sackstein



SUMMER COURSE-17TH EDITION
FROM 10-14 OF JULY-LOS ALCÁZARES
MURCIA

Place: Hotel 525. - Calle del Río Borines 58 - 30710 Los Alcázares, Murcia

Duration: 30 hours - Registration Deadline: 5/07/2022

More Information and Registration Form:

www.um.es/unimar

UNIVERSIDAD DE
MURCIA



 **Terav** ^{ISCIII}
Red Española de Terapias Avanzadas - TerCel



Sociedad Española de
Hematología y Hemoterapia

DIRECTORS

Prof. José María Moraleda Jiménez.

Professor of Medicine. University of Murcia. Head Dpt. of Hematology and HSCT Program. Institute of Biomedical Research of Murcia - IMIB, Vigen de la Arrixaca University Hospital. Murcia, Spain.

Prof. Robert Sackstein.

MD,PhD. Senior Vice-President for Global Medical Affairs, Florida International University, Miami, FL Professor Emeritus, Harvard Medical School, Boston, MA.

AIMS

The aim of this course is the diffusion of knowledge about Advanced Therapies (gene therapy, somatic cell therapy, tissue engineering), and their therapeutic possibilities, with a special emphasis on their current applications in the field of genetic and neoplastic diseases as well as regenerative Medicine.

The course is intended to provide a review of current clinical applications of advanced therapies, a forum for scientific discussion with basic and clinical researchers that are experts in advanced therapies, and to present the achievements and challenges in this exciting and growing area of medical science.

Specific Course Objectives are:

To analyze in depth the different types of advanced therapies and the biological properties that support its therapeutic potential.

To learn about advances in hematopoietic stem cell transplantation, as the first immune-cellular therapy to cure genetic and malignant diseases.

To learn the advances in gene therapy, immunotherapy with CAR cells, somatic cell therapies, and how to manufacture advanced cell therapies compliant with regulatory science to treat patients.

To present and discuss the current status of preclinical studies and clinical trials of advanced therapies to cure diseases.

PLACE: HOTEL 525

LOS ALCÁZARES - MURCIA

Duration: 30 hours

Language: Spanish/English

Max enrollment: 200 students

Prize: 175€

Online: 85€

TARGET GROUP

Collective of health professionals and students: haematologists and physicians of any other specialty interested in advanced therapies, as well as health professionals, researchers and university students of health and health-related degrees. Other target groups are not professionals: groups involved who benefit from the information being shared in this course, such as patient associations, industry, companies, students, as well as other people and sectors of the population, with curiosity in the scientific field, who want obtain objective, critical and useful information to lay the foundations of appropriate knowledge.



REGISTRATION RULES

Please complete the Registration Form at the official website:

<https://www.um.es/unimar>

Payment Methods:

Credit Card (Website).

Cash: only in person. Download the Payment form. Fill in the form and return it with your payment to Universidad Internacional del Mar. Banks: Sabadell, Bankia, Cajamar and Santander.

Once payment is made you will be enrolled in the course. Students must be enrolled and have a regular attendance to obtain the diploma or its equivalent.

MONDAY-10-JULY

HEMATOPOIETIC STEM CELL TRANSPLANTATION. THE FIRST SUCCESSFUL CELL THERAPY

09:00-09:30 h. Opening Session

Hon. Mr. Juan José Pedreño.
Health Council of the Autonomous Community of the Region of Murcia. Spain.

Hon. Mr. José Luján Alcaráz.
Rector of the University of Murcia. Spain.

Hon. Mr. Cristóbal Belda Iniesta.
Director Institute of Health Carlos III (ISCIII). Madrid. Spain.

Morning Session

Chair: Prof. José M^a. Moraleda & Prof. Agustín Zapata. Coordinators Ricors-TeraV.

09:30-10:00 h. Hematopoietic Stem Cell (HSC): Biological characteristics.

Prof. Agustín Zapata. Professor of Cell Biology. Complutense University of Madrid, Spain.

10:00-10:30 h. Hematopoietic Stem Cell (HSC): Sources and procurement. The transplant lab.

Prof. Miguel Blanquer. University of Murcia. Hematology Department and HSCT Program Virgen de la Arrixaca University Hospital. IMIB-Pascual Parrilla. Murcia, Spain.

10:30-11:00 h. Coffee break

11:00-11:30 h. Hematopoietic stem cell transplantation: the first cellular therapy.

Prof. José M^a. Moraleda. Professor of Medicine. University of Murcia. Head Dpt. of Hematology and HSCT Program Virgen de la Arrixaca University Hospital. IMIB-Pascual Parrilla. Murcia, Spain.

11:30-12:00 h. The stem cell immune effects. Graft versus host disease and GVL effects.

Prof. José Antonio Pérez Simón. Professor of Hematology. Head Hematology Dpt. Institute of Biomedicine, Virgen del Rocio University Hospital. University of Sevilla, Spain.

12:00-12:30 h. Endothelial-cell related complications of SCT.

Prof. Rafael Duarte. Head Hematology Dpt. of University Hospital Puerta de Hierro. Madrid, Spain.

12:30-13:00 h. Immunotherapy for pediatric tumors, from NK cells to CAR T.

Prof. Lucía Fernández Casanova. National Center for Oncology Research (CNIO). Madrid. Spain.

13:00-13:30 Discussion.

13:30-15:30 h. Lunch.

Afternoon Session

Chair: Prof. Robert Sackstein. Professor Emeritus, Harvard Medical School. Boston, MA. Senior Vice-President for Global Medical Affairs, Florida International University. Miami, FL. USA.

15:30-16:00 h. Transplantation in Europe. Main indications. Present and future.

Prof. Anna Sureda. President EBMT. Head Department of Hematology. ICO. Barcelona, Spain.

16:00-16:30 h. Improving SCT results. Are there any magic receipts?

Prof. Alejandro Madrigal. Professor of Haematology, UCL Cancer Institute. Honorary Consultant, Royal Free NHS Trust UCL Country Ambassador for México. London, UK.

16:30-17:00 h. Debate: Unsolved questions about HSCT. Discussants (10 min. each and discussion):

Chair: Prof. Robert Sackstein & Jose M. Moraleda.

- **What is the place of HSCT in the era of new immunotherapies?** **Prof. Jose A. Pérez Simón & Prof. Rafael Duarte.** Hematology Dpt. Virgen del Rocio. Sevilla // Hematology Dpt. Puerta de Hierro. Madrid. Spain.
- **Do we still need the search for HLA-identical SCT donors?** **Prof. Alejandro Madrigal.** Chair of Hematology. The Royal Free and University College Medical School. London, UK.

17:00-18:00 h. Welcome students' reception.



TUESDAY-11-JULY

GENE THERAPY:

SCIENTIFIC BACKGROUND AND THERAPEUTIC USE

Morning Session

Chairs: Prof. Juan Bueren. Division of Hematopoiesis and Gene Therapy. CIEMAT.
Prof. Francisco Martín Molina. GENYO. Head Gene and Cell Therapy Group.

Session I: Tools for genetic modifications in gene therapy strategies.

09:00-09:30 h. *Gene editing for gene therapy applications.*

Prof. Oscar Quintana. Cell Technology Unit. CIEMAT, CIBERER and IIS Fundación Jiménez Díaz. Madrid. Spain.

09:30-10:00 h. *Basis of gene therapy with adenoassociated vectors.*

Prof. Gloria González-Aseguinolaza. Applied Medical Research Center (CIMA). Spain.

10:00-10:30 h. *Basis of gene therapy with lentiviral vectors.*

Prof. María Tristán. LentiStem Biotech. Spain.

10:30-11:00 h. *Applications of nanotechnology to gene therapy.*

Prof. Ramón Martínez-Mañez. Polytechnic University of Valencia. CIBER-BBN. Spain.

11:00-11:30 h Coffee break

Session II: Towards the gene therapy of monogenic diseases.

11:30-12:00 h. *First gene therapy for hereditary retinal dystrophy. Contributions to clinical practice.*

Prof. Almudena de Pablo. Retina section of the Ophthalmology Service. Hospital October 12. Madrid. Spain.

12:00-12:30 h. *Advances in the gene therapy of hemophilia.*

Prof. Victor Jiménez Yuste. Hematology Service. La Paz University Hospital. Madrid. Spain.

12:30-13:00 h. *Gene Therapy in patients with Leukocyte Adhesion Deficiency Type I (LAD-I).*

Prof. Elena Almarza. Associate Director, Principal Scientist. Rocket Pharmaceuticals. Spain.

13:00-13:30 h. *Advances in the gene therapy of MNGIE.*

Prof. Jordi Barquinero. Vall d'Hebron Institut de Recerca. Barcelona. Spain.

13:30- 15:30 h. Lunch.

Afternoon Session

Cancer Virotherapy and gene therapy.

Chairs: Prof. Africa González-Murillo. Niño Jesús Hospital. Madrid. Spain. Prof. Ramón Alemany. Institut Català Oncologia. Barcelona. Spain.

15:30-16:00 h. *Basis of oncolytic virotherapy*

Prof. Cristina Fillat. Group Leader Gene Therapy and Cancer. IDIBAPS; CIBERER. Spain.

16:00-16:30 h. *Oncolytic Virotherapy with adenoviral viruses.*

Prof. Marta Alonso. Applied Medical Research Center (CIMA). Spain.

16:30-17:00 h. *Patient interview: The patient view.*

D. Secundino Sacristán. Executive member of ALHETA (Spanish Association for the Fight against Hemoglobinopathies and Thalassemias). National Merit 2023 of the blood donor. Valladolid. Spain.



WEDNESDAY-12-JULY

ANTITUMOR IMMUNOTHERAPY. CHIMERIC ANTIGEN RECEPTOR (CAR) CELLS

Morning Session

Chair: Prof. Felipe Prósper. Professor of Hematology. University of Navarra.

Prof. Manel Juan. Professor of Immunology. University of Barcelona.

09:00-09:30 h. CAR T- cells. Biological principles and academic manufacture.

Prof. Manel Juan. Head Immunology Dpt. Hospital Clinic. Professor of immunology. University of Barcelona, Spain.

09:30-10:00h. CAR T- cells for the treatment of Leukemia.

Prof. Alvaro Urbano. Professor of Medicine. University of Barcelona. Hospital Clinic. Institut Josep Carreras. Barcelona, Spain.

10:00-10:30 h. CAR T- cells for the treatment of Multiple Myeloma.

Prof. Carlos Fernández de Larrea. Professor of Medicine. University of Barcelona. Hospital Clinic. Institut Josep Carreras. Barcelona, Spain.

10:30-11:00 h. Coffee break

11:00-11:30 h. CAR T- cells for the treatment of Lymphoma.

Prof Javier Briones. Director Clinical Hematology. Hospital Sat Pau. Barcelona. Professor of Medicine. UAB. Barcelona. Spain.

11:30-12:00 h. Mechanisms of toxicity and resistance to CAR T- cells. How to overcome it.

Prof. Felipe Prósper. Professor of Medicine. Head Hematology Dpt. CUN. University of Navarra. Pamplona. Spain.

12:00-12:30 h. CAR NK. Scientific background and clinical applications.

Prof. Cristina Eguizabal. Principal investigator Cell Therapy, Stem Cells and Tissues research group. Biocruces Bizkaia. Osakidetza Basque Center Transfusion and Human Tissues. Bilbao. Spain.

12:30-13:00 h. Advances in CAR immunotherapies generated by our group in TERA.V.

Prof Pablo Menendez. Research Professor. ICREA. Jose Carreras Research Institute. University of Barcelona. Spain.

13:00-13:30h. Predictive factors for response to immunotherapy.

Prof. Ana Alarcón Tomás. Dpt Hematology. University Hospital Puerta de Hierro. Madrid. Spain.

13:30-15:30 h. Lunch.

Afternoon Session

15:30-17:00 h. Special session: CAR T- cells in Spain, were we do stand?

Chair: Prof. Anna Sureda. President EBMT. Head Hematology Service Hospital Duran I Reinalds. Barcelona. Spain. **Prof. Joaquín Martínez López.** Professor of Medicine. Head Hematology Dpt. University Hospital 12 Octubre. (i+12). Complutense University. Madrid. Spain.

15:30-16:00 h. The Spanish academic CAR T- cells model.

Prof Álvaro Urbano Izpisua. Professor of Medicine. University of Barcelona. Hospital Clinic. Institut Josep Carreras. Barcelona, Spain.

16:00-16:30 h. The view of the ONT about Advanced Therapies in Spain and its regulation.

Dr. Dolores Hernández Maraver. Medical director. National Transplant Organization (ONT). Madrid. Spain.

16:30-17:00 h. ATMPs: how to get them affordable maintaining the National Health System.

Prof César Hernández. General Sub-Director of Common Portfolio of Services of the National Health and Pharmacy System. Ministry of Health. Spain.



THURSDAY-13-JULY

SOMATIC CELL THERAPY AND TISSUE ENGINEERING: UPTODATE

Morning Session

Chairs: **Prof. Agustín Zapata.** Professor of Cell Biology. Complutense University of Madrid, Spain.
Prof. Damián García-Olmo. Professor of Surgery. Autonomous University of Madrid.

09:00-9:20 h. MSC biological properties and different sources: the case of umbilical cord MSC.

Prof. Sergi Querol. Director. Banc de Sang I Teixits. Barcelona. Spain.

09:20-10:10 h. The glicobiology of the main adult stem cells: HSC and MSC.

Prof. Robert Sackstein. Professor Emeritus, Harvard Medical School. Boston, MA. Senior Vice-President for Global Medical Affairs, Florida International University. Miami, FL. USA.

10:10-10:30 h. Genetically modified MSC to improve anti-inflammatory function.

Prof. Rosa María Yañez González. Hematopoietic Innovative Therapies Division. CIEMAT. University of Madrid. Spain.

10:30-11:00 h. Coffee break

11:00-11:20 h. MSC therapy for the treatment of Graft versus host disease: Where do we stand?

Prof. Fermín Sánchez-Guijo. Professor of Hematology. University Hospital of Salamanca. University of Salamanca, Spain.

11:20-11:40 h. MSC therapy for the treatment of osteoarticular diseases.

Prof. José Lamo de Espinosa. Professor of Orthopedics, University of Navarra. Spain.

11:40-12:00 h. MSC therapy for the treatment of critical limb ischemia. The NOMA trial.

Prof. Mariano García Arranz. Senior Research. Fundación Jimenez Diaz. Autonomous University of Madrid. Spain.

12:00-12:20h. Cell therapy and bioengineering for skin diseases.

Prof. Álvaro Meana. Doctor of medicine and surgery. Oviedo University. Tissue Engineering Unit of the Asturias Community Blood and Tissue Center. Spain.

12:20-12:40 h. MSC therapy for the treatment of ALS.

Prof. Salvador Martínez Pérez. Professor of Human Anatomy and Embryology. University Miguel Hernandez-CSIC. Elche. Spain.

12:40-13:00 h. Somatic cell therapy for the treatment of Parkinson.

Prof. Juan Jose Toledo. Professor of Physiology. Institute of Biomedical Research of Seville. IBiS, Virgen del Rocío Hospital/CSIC/University of Sevilla. Spain.

13:00-13:10 h. Discussion.

13:30-15:30 h. Lunch.

Afternoon Session

Chair: **Prof. Fermín Sanchez-Guijo.** Professor of Medicine. University of Salamanca. Spain.

15:30-16:00 h. Cell Therapy research in Surgery. Real life results.

Prof. Damián García-Olmo. Professor of Surgery. Chief of Department of Surgery. Fundación Jimenez Diaz University Hospital. Autonomous University of Madrid, Spain.

16:00-16:30 h. MSC indications, activity and monitoring in Europe.

Prof. Christian Chabannon. Professor of Medicine in Cell Biology at Aix Marseille Université (AMU) School of Medicine, President of the CME of "Institut Paoli Calmette". Marseille, France.

16:30-17:00 h. Discussion.



FRIDAY-14-JULY

ADVANCED THERAPIES MANUFACTURE AND INNOVATION: A REAL CHALLENGE

Morning Session

Chairs: Prof. Jose M. Moraleda. Ricors-TeraV Coordinator. Prof. Anna Sureda. Head Hematology Service Hospital Duran I Reinal. Barcelona. Spain.

09:00-09:20 h. *Production of cells in GMP facilities: What is new in 2023?*

Prof. María Eugenia Fernández-Santos. PhD. Technical Director of the Cell Production Unit. Gregorio Marañón University Hospital. Research Institute Gregorio Marañón. Madrid. Spain.

09:20-09:40 h. *The viral production for gene therapy and advanced therapies.*

Prof. Josep Canals. Associate Professor University of Barcelona. Head stem cells and regenerative medicine research Unit. Dpt of Biomedicine. Barcelona. Spain.

09:40-10:00 h. *Innovative therapies based on cell and tissue donation.*

Prof. Joaquim Vives. Director de R&D, XCELIA - Banc de Sang i Teixits. Barcelona. Spain.

10:00-10:20 h. *The molecular biology of pericytes in disease: a new target for advanced therapies.*

Prof. Rut Valdor. Cell Therapy Unit and HSCT Program. IMIB-Pascual Parrilla. University of Murcia, Spain.

10:20-10:30 h. Discussion.

10:30-11:00 h. Coffee break.

11:00-11:20 h. *CD84: a novel target for CAR T-Cell Therapy for Acute Myeloid Leukemia.*

Prof. Nela Klein-González. Research Scientist. Hospital Clinic de Barcelona. Spain.

11:20-12:40 h. *Special Session: Networks of ATMPs in Europe.*

Chair: Prof. Anna Sureda. President EBMT. Head Hematology Service Hospital Duran I Reinal. Barcelona. Spain.

11:20-11:40 h. *GoCAR-T Initiative.*

Prof. Christian Chabannon. Professor of Medicine in Cell Biology at Aix Marseille Université (AMU) School of Medicine, President of the CME of "Institut Paoli Calmette". Marseille, France.

11:40-12:00 h. *Dutch ATMP initiatives: DARE-NL and GMP simulator – ICAT.*

Prof. Trudy Straetemans. Associate Professor. University Medical Centre Utrecht. The Netherlands.

12:00-12:20 h. *The HOVON161 clinical trial. A disruptive ATMP academic trial.*

Prof. Tom Van Meerten. Prof. Hematology. University Medical Center Groningen. The Netherlands.

12:20-12:40 h. *Advanced Therapies Network in Spain (Ricors-TeraV).*

Prof. Jose M. Moraleda. Ricors-TeraV Coordinator. Dpt. of Hematology and HSCT Program Virgen de la Arrixaca University Hospital. IMIB-Pascual Parrilla. University of Murcia, Spain.

12:40-13:00 h. Discussion.

13:00-13:30 h. *Hospital exemption: a necessary tool for academic ATMPs.*

Prof. Sol Ruíz Antúnez. Head of the Unit of Biotechnology and Advanced Therapies at EMA. AEMPS. Madrid, Spain.

13:30-15:30 h. Lunch.

Afternoon Session

15:30-16:30 h. Debate session:

The unsolved problems of academic advanced cell manufacture. How to improve the accessibility and affordability of the ATMPs.

Chairs: Prof. Jose M Moraleda, Prof. Agustín Zapata & Prof. Robert Sackstein.

Discussants (10min. each and discussion):

- **Prof. Sol Ruíz Antúnez.** Head of the Unit of Biotechnology and Advanced Therapies at EMA. Madrid, Spain.
- **Prof. Anna Sureda.** Head Hematology Service Hospital Duran I Reinal. Barcelona. Spain.
- **Prof. Natividad Cuende.** Andalusian Transplant Coordination.
- **Prof. Natacha Bolaños.** Patient association. Lymphoma coalition.

17:00-17:30 h. *Closing ceremony and awarding of certificates.*

Prof. Cristobal Belda. Director. Institute of Health Carlos III. Ministry of Science and Innovation. Madrid. Spain.



SUMMER COURSE – 2023

ADVANCED THERAPIES

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