

Programme

Monday 19 June

08:30-09:00 Registration	
09:00-10:00 Session 1: Inaugural session	Room: Sala Ciudadela
Chairs: Gloria Gonzalez Asequinolaza , <i>University of Navarra, Pamplona</i> , Ander Izeta , <i>Biodonostia, San Sebastian</i>	
09:00 Gloria Gonzalez Asequinolaza , <i>University of Navarra, Pamplona</i> Opening and welcome	
09:15 Ignacio Melero , <i>University of Navarra, Pamplona</i> INV01 Opening Keynote	
10:00-11:30 Session 2: Gene editing	Room: Sala Ciudadela
Chairs: Javier Molina , <i>GENYO</i> , Juanro Rodriguez Madoz , <i>University of Navarra, Pamplona</i>	
10:00 Toni Cathomen , <i>University of Freiburg</i> INV02 What can we learn from meaningful assessments of on- and off-target effects in genome editing?	
10:30 Natalia Artigas , <i>Integra Therapeutics S.L.</i> OR01 Characterization and deployment in vivo of FiCAT genome writer	
10:45 Araceli Aguilar-González , <i>GENyO/ Universidad de Granada / Junta de Andalucía</i> OR02 CRISPNA, a new tool for genome editing and diagnosis	
11:00 Karim Benabdellah , <i>GENyO/ Universidad de Granada / Junta de Andalucía</i> OR03 Breaking Barriers and Building Bridges: The Impact of COST Action GenE-Humdi in Advancing Genome Editing for Human Disease Treatment	
11:15 Laura Torella , <i>FIMA, Universidad de Navarra, Pamplona</i> P047 AAV-SaCas9 Nickase targeting Hao1 is a safe and efficient therapy for Primary hyperoxaluria type 1 with a low AAV integration rate	
11:20 Martin Bilbao Arribas , <i>CIMA, Universidad de Navarra, Pamplona</i> P050 Developing new mouse gene editing tools to assess Pyruvate Kinase Deficiency stem cell gene editing in a mouse model of the disease	
11:25 Sara Veiga , <i>University of Santiago de Compostela</i> P054 Generation of brain organoids and zebrafish models to determine and reverse ASD-related phenotypes	
11:30-12:00 Coffee Break	

<p>12:00-13:30 Room: Sala Ciudadela Session 3a: Gene & cell therapy of metabolic diseases</p> <p>Chairs: Virginia Haurigot, <i>Spark Therapeutics</i>, Fatima Bosch, <i>UAB, Barcelona</i></p> <p>12:00 Gloria Gonzalez-Aseguinolaza, <i>University of Navarra, Pamplona</i> INV03 Gene therapy for liver metabolic disorders</p> <p>12:30 Nicholas Weber, <i>Vivet-Therapeutics</i> OR04 Effective Readministration of AAV Gene Therapy Prevents Tumorigenesis in Infant PFIC3 Mice, a Mouse Model Prone to Hepatocellular Carcinoma</p> <p>12:45 Sergio Muñoz, <i>Universidad Autónoma de Barcelona</i> OR05 Treatment of infantile-onset Pompe disease in a rat model with muscle-directed AAV Gene Therapy</p> <p>13:00 Juan Ruiz, <i>Forge Biologics Inc</i> OR06 FBX-101 Clinical program for Patients with Krabbe Disease – Updates on the RESKUE & REKLAIM Phase I/II Gene Therapy Clinical Trials</p> <p>13:15 Oihana Murillo, <i>CIMA, Universidad de Navarra, Pamplona</i> P042 Therapeutic efficacy of the combination of VTX-801 and D-Penicillamine for Wilson's disease</p> <p>13:20 Daniel Jerico, <i>CIMA, Universidad de Navarra, Pamplona</i> P060 mRNA-based therapy in a new model of variegate porphyria in rabbits</p> <p>13:25 Angie Molina, <i>CIMA, Universidad de Navarra, Pamplona</i> P044 Improvement of progressive familiar intrahepatic cholestasis type 2 (PFIC2) via AAV-mediated hepatic BSEP expression in mice</p>	<p>12:00-13:30 Room: Luneta Session 3b: Oncolytic viruses & cancer immunotherapy</p> <p>Chairs: Alvaro Morales, <i>Instituto de Salud Carlos III</i>; Cristian Smerdou, <i>CIMA, Pamplona</i></p> <p>12:00 Marta Alonso, <i>University of Navarra, Pamplona</i> INV04 Virotherapy for the Treatment of Pediatric Brain Tumors</p> <p>12:30 Maria Tristán-Manzano, <i>Lentistem</i> OR07 Lentiviral vectors for inducible, transactivator-free advanced therapy medicinal products: application to CAR-T cells</p> <p>12:45 Marc Otero, <i>IDIBAPS, Barcelona</i> OR08 KPC pancreatic cancer cells established from a murine pancreatic tumor support human adenovirus replication in vitro and in vivo</p> <p>13:00 Ana Judith Perisé Barrios, <i>Universidad Alfonso X el Sabio, Villanueva de la Cañada</i> OR09 Canine high-grade gliomas treated with the oncolytic adenovirus ICOCAV15: a preclinical model</p> <p>13:15 Ana del Cañizo Lázaro, <i>Universitat de Barcelona</i> P083 Improving poxvirus-mediated antitumor immune responses by deleting viral cGAMP-specific nuclease</p> <p>13:20 Pau Fiol, <i>IDIBELL-ICO, Barcelona</i> P081 Oncolytic adenovirus coding for MICA enhances immune responses against tumors</p> <p>13:25 Patricia García Rodríguez, <i>Institut de Salut Carlos III</i> P086 Oncolytic virus and CAR-T cells combination therapy to treat solid tumors.</p>
<p>13:30-14:30 Lunch</p>	
<p>14:30-16:00 Poster session 1</p>	

<p>16:00-17:30 Room: Sala Ciudadela Session 4a: Gene & cell therapy for haematological disorders Chair: Raul Torres, CNIO / CIEMAT, Susana Navarro, CIEMAT / CIBERER / IIS-FJD</p> <p>16:00 Paula Rio, CIEMAT, Madrid INV05 Therapeutic Gene Editing in Hematopoietic Stem Cells: Meeting the Challenge in Fanconi Anemia</p> <p>16:30 Maria Fernandez Garcia, Ciemat/CIBERER, Madrid OR10 Improved efficacy of mesenchymal stromal cells stably expressing CXCR4 and IL-10 in a xenogeneic graft versus host disease mouse model</p> <p>16:45 Manuel Palacios Pérez, Ciemat/CIBERER, Madrid OR11 Development of a gene editing approach for the treatment of RPL5-deficient Diamond-Blackfan anemia patients</p> <p>17:00 Beatriz Olalla, Ciemat/CIBERER, Madrid OR12 Gene targeting of hematopoietic stem and progenitor cells by prime editing</p> <p>17:15 Iris Ramos Hernández, GENyO-Pfizer / Universidad de Granada / Junta de Andalucía P039 HSPCs targeted insertion in CX3CR1 enhances myeloid expression of integrated cassettes</p> <p>17:20 Guillermo Güenechea Amurrio, CIEMAT/CIBERER, Madrid P024 Lentiviral-mediated gene therapy restores telomere homeostasis in ex vivo assays on dyskeratosis congenita-like CD34+ cells</p> <p>17:25 Irene Romayor, BioCruces/Hospital Universitario Cruces P022 Disease-corrected hematopoietic progenitors from APDS2 syndrome derived iPS cells</p>	<p>16:00-17:30 Room: Luneta Session 4b: Regenerative medicine Chair: Manu Mazo, Universidad de Navarra, Pamplona, Ander Izeta, Biodonostia, San Sebastian</p> <p>16:00 Antonella Consiglio, Idibell, Barcelona INV06</p> <p>16:30 Leire Gardeazabal, Biodonostia Institute OR13 Characterisation of cutaneous wound healing of aged Sox2EGFP mice</p> <p>16:45 Julia López de Andrés, University de Granada OR14 Using patient-derived extracellular matrix for customized tissue substitutes: Advancements in cellular and tissular therapies</p> <p>17:00 Ainhoa Irastorza Lorenzo, Biodonostia Institute OR15 Development of novel muscle bioink based on decellularised tissue and myogenic precursor cells for the treatment of stress urinary incontinence</p> <p>17:15 Andrea Sánchez Bueno, CIMA, Universidad de Navarra, Pamplona P094 Composite human cardiac minitissues are a representative 3D model of anthracycline cardiotoxicity</p> <p>17:20 Paula Barlabé, CIMA, Universidad de Navarra, Pamplona P095 Generation of heart and vascular system from pluripotent stem cells by blastocyst complementation.</p> <p>17:25 Juan Antonio Marchal, CIBM, Universidad de Granada, Granada P098 Bioinks based on decellularised extracellular matrix from cells for osteochondral grafts</p>
<p>17:30-18:00 Coffee Break</p>	
<p>18:00-19:00 Room: Sala Ciudadela Session 5: Keynote Chair: Juan Bueren, CIEMAT, Madrid</p> <p>18:00 Felipe Prosper, Universidad de Navarra INV07 Understanding mechanisms of response and resistance of CAR-T cells in hematological malignancies</p>	
<p>19:30-21:30 Welcome reception</p>	

Tuesday 20 June

08:00-08:30 Registration	
<p>08:30-10:00 Room: Sala Ciudadela</p> <p>Session 6a: In vivo gene & cell therapy non viral</p> <p>Chairs: Maria Jesus Garrido, <i>Universidad de Navarra, Pamplona</i>; Kepa Berraondo, <i>Program of Immunology and Immunotherapy</i></p> <p>08:30 Gemma Navarro, <i>Curevac</i> INV08 Optimizing RNA therapeutics delivery</p> <p>09:00 Eric Rovira Barreira, <i>CIMA, Universidad de Navarra, Pamplona</i> OR16 Identification of novel sequences to regulate gene expression in response to approved drugs for application in gene therapy.</p> <p>09:15 Lydia Alvarez-Erviti, <i>Centro de Investigación Biomédica de La Rioja (CIBIR)</i> OR17 Delivery of shRNA minicircles by extracellular vesicles to halt Parkinson's disease neurodegeneration</p> <p>09:30 Noelia Silva Pilpich, <i>CIMA, Universidad de Navarra, Pamplona</i> OR18 Targeting the PD-1/PD-L1 pathway with nanobodies encoded in self-amplifying RNA vectors induce potent antitumor responses</p> <p>09:45 Yaiza Jimenez Martinez, <i>Universidad de Granada</i> P051 IdrB gene: elucidating mechanism of action of a suicide gene.</p> <p>09:50 Natalia Hernández Bellido, <i>I3A, University of Zaragoza</i> P064 Non-viral delivery of microRNA therapies for cardiac dysfunction using DNA nanostructures</p> <p>09:55 Luis Sendra, <i>Instituto de Investigación Sanitaria La Fe</i> P065 Hepatic hydrofection of naked hIL10 gene in pig to study its immunomodulatory effect in liver transplantation scenario</p>	<p>08:30-10:00 Room: Luneta</p> <p>Session 6b: Gene editing II</p> <p>Chairs: Laura Torella, <i>FIMA, Universidad de Navarra, Pamplona</i>; Sandra Rodriguez, <i>Spanish National Cancer Centre (CNIO), Madrid</i></p> <p>08:30 Eliana Ruggiero, <i>HSR, Milan</i> INV09 Engineering T lymphocytes for the treatment of hematological and solid tumors</p> <p>09:00 Irene Peña Gutierrez, <i>Ciemat/CIBERER, Madrid</i> OR19 Mimicking FA mutations in hematopoietic stem and progenitor cells by gene editing approaches</p> <p>09:15 Alex Bassons Bascunana, <i>Ciemat/CIBERER, Madrid</i> OR20 Highly efficient non-viral gene editing approaches applicable to all COL7A1 mutations known.</p> <p>09:30 Ignacio Perez de Castro, <i>Instituto de Salud Carlos III</i> OR21 Investigating the potential of different gene therapy strategies for treating LMNA-associated congenital muscular dystrophy: varied outcomes depending on the pathological contexts</p> <p>09:45 Simonas Valiuska, <i>University of Barcelona</i> P052 Combinatorial effect of Polypurine Reverse Hoogsteen against MYC and KRAS oncogenes in the human PC-3 cancer cell line</p> <p>09:50 Jose Carlos Segovia-Sanz, <i>CIEMAT/CIBERER, Madrid</i> P050 Developing new mouse gene editing tools to assess Pyruvate Kinase Deficiency stem cell gene editing in a mouse model of the disease</p> <p>09:55 Kristina Pavlovic, <i>GENyO-Pfizer / Universidad de Granada / Junta de Andalucía</i> P025 Memory Universal CD19-CAR T cells: a new strategy against B-cell malignancies</p>
10:00-10:30 Coffee Break	

10:30-12:00

Room: Sala Ciudadela

Session 7: Engineered cells for cancer immunotherapy I

Chairs: **Javier Garcia-Castro**, *Instituto de Salud Carlos III*; **Francisco Martin**, *GENYO, Granada*

10:30 **Luis Alvarez-Vallina**, *H12O-CNIO Cancer Immunotherapy Clinical Research Unit Clinical Research Programme, Hospital Universitario 12 de Octubre (H12O), Spanish National Cancer Research Centre (CNIO)*

INV10 Novel T cell-redirecting strategies for enhanced cancer immunotherapy: Dynamic targeting.

11:00 **Diego Sanchez Martinez**, *Josep Carreras Institute and CIBERINFEC, Barcelona*

OR22 A novel fratricide-resistant CAR-T cell immunotherapy for T-ALL

11:15 **Alba Rodriguez Garcia**, *IDIBAPS, Barcelona*

OR23 Sensitivity of CAR-T cells to PD-1/PD-L1-mediated inhibition is modulated by CAR affinity

11:30 **Pedro Luis Justicia Lirio**, *Lentistem*

OR24 Development of first-in-class doxycycline-inducible IL-18 secreting CAR-T cells targeting aggressive tumors

11:45 **Claudia Augusta Di Trani**, *CIMA, Universidad de Navarra, Pamplona*

P082 Intra-tumoral delivery of mRNA encoding IL-12-fused to diabodies targeting CSF1R and PD-L1 exert potent anti-tumor efficacy while restraining systemic exposure to IL-12.

11:50 **Catalina Barceló**, *IDIBAPS, Barcelona*

P005 Competition of co-transduced CARs for cellular resources in dual-targeting CAR T cells

11:55 **Paula Heredia Velázquez**, *GENYO-Pfizer / Universidad de Granada / Junta de Andalucía*

P009 Improving exosome production and immunogenicity for more effective CAR T cell therapy

12:00-13:00

Room: Sala Ciudadela

Session 8: The future of advanced therapies in Spain

Chairs: **Paula Rio**, *CIEMAT, Madrid*; **Ander Izeta**, *Biodonostia, San Sebastian*

12:00 **Raul Torres**, *CIEMAT/CIBERER, Madrid*

INV11a Desarrollo de Terapias Avanzadas en España

12:30 **Nerea Zabaleta**, *Harvard Medical School / Mass Eye and Ear*

INV11b Rational engineering of AAV libraries reveals capsid residues that determine tropism

13:00-14:00

Lunch

14:00-15:30

Poster session 2

15:30-17:00

Room: Sala Ciudadela

Session 9: Engineered cells for cancer immunotherapy II

Chairs: **Ruben Hernández**, *Universidad de Navarra*; **Sonia Guedan**, *IDIBAPS, Barcelona*

15:30 **Cristina Puig-Saus**, *UCLA*

INV12 Engineering a potent T cell response against solid tumors

16:00 **Mireia Bachiler**, *IDIBAPS, Barcelona*

OR25 ARI0003: Co-transduced CD19/BCMA dual-targeting CAR-T cells for the treatment of non-Hodgkin lymphoma

<p>16:15 Paula Rodríguez Márquez, <i>CIMA, Universidad de Navarra, Pamplona</i> OR26 Prolonged haematologic toxicity after BCMA CAR-T cell treatment: from clinical data to ex-vivo studies at a single center</p> <p>16:30 Andrea Lopez, <i>CIEMAT/CIBERER, Madrid</i> OR27 Trogocytosis in CAR-T cells against HNSCC cell lines</p> <p>16:45 Angela Covo, <i>CIMA, Universidad de Navarra, Pamplona</i> P001 Semliki Forest virus-based vaccines encoding CAR antigens to foster the activation, expansion and antitumor efficacy of CAR-T cells.</p> <p>16:50 Lorea Jordana, <i>CIMA, Universidad de Navarra, Pamplona</i> P006 Identification of molecular mechanisms governing CAR-T cell response in MM patients using single cell transcriptomics</p> <p>16:55 Patricia Garcia Rodriguez, <i>Instituto de Salud Carlos III, Madrid</i> P011 CAR T therapy efficacy in hypoxic osteosarcoma spheroids.</p>	
<p>17:00-17:30 Coffee Break</p>	
<p>17:30-19:00 Room: Sala Ciudadela Session 10a: Manufacturing Chairs: Maria Eugenia Fernandez, <i>Fundacion para la Investigacion Biomedica del Hospital G. Maranon, Madrid</i>; Begoña Diez, <i>CIEMAT/CIBERER/IISFJD, Madrid</i></p> <p>17:30 Gemma Fernández de Miguel, <i>Pharmaceutical Inspection and Enforcement Department</i> INV13 Global Overview of Advanced Therapy Medicines (ATMPs) Manufacturing</p> <p>18:00 Gloria Carmona, <i>Junta de Andalucía</i> INV14 Quality Controls in Open and Closed Processes</p> <p>18:30 Maria Camanyes Novellón, <i>IDIBAPS, Barcelona</i> OR28 Quality control establishment for gene therapy medicinal products</p> <p>18:45 Silvia Torrents Zapata, <i>Banc de Sang i Teixits</i> P070 Optimized reagents for immunopotency assays on mesenchymal stromal cells for clinical use</p> <p>18:50 Ana Carreras González, <i>Viralgen Vector Core</i> P072 Addressing the Challenges of Capillary Electrophoresis for AAV Purity Characterization</p> <p>18:55 Katherine Orange-Bromehead, <i>TAAV Manufacturing Solutions</i></p>	<p>17:30-19:00 Room: Luneta Session 10b: Gene & cell therapy for neurological disorders Chairs: Carmen Unzu, <i>CIMA, Universidad de Navarra</i>; Miguel Chillón, <i>Vall d'Hebron University Hospital Foundation, Barcelona</i></p> <p>17:30 Juan Jo Toledo Aral, <i>University of Sevilla</i> INV16 Antiparkinsonian cell therapy: pros and cons of the coronavirus pandemic.</p> <p>18:00 Sara Marcó Costa, <i>CBATEG - Universitat Autònoma de Barcelona</i> OR29 A new gene therapy approach to treat Niemann-Pick Type C2 disease</p> <p>18:15 Anna Mateu Bosch, <i>IDIBAPS, Barcelona</i> OR30 GCDH gene replacement therapy ameliorates glutaric aciduria type I phenotype in mice.</p> <p>18:30 Sergi Verdés, <i>UUniversitat Autònoma de Barcelona (UAB)</i> OR31 A novel gene therapy approach for ALS by overexpressing in muscles the pleiotropic chronokine α-Klotho</p> <p>18:45 Eulàlia Segur-Bailach, <i>IDIBAPS, Barcelona</i> P038 Substrate reduction gene therapy rescues glutaric aciduria type I in mice.</p> <p>18:50 Ruben Hernandez-Alcoceba, <i>CIMA, Universidad de Navarra, Pamplona</i> P026 Strategies to obtain preferential transgene expression in GABAergic neurons using adenoviral vectors</p>

<p>P075 TAAV-manufactured enzymatic DNA: an efficient technology to overcome rAAV manufacturing challenges</p>	<p>18:55 Joan Roig Soriano, <i>Universitat Autònoma Barcelona</i> P028 AAV9-Mediated expression of secreted Klotho reduced several aging-associated phenotypes and increased longevity</p>
<p>19:00-20:00 Room: Sala Ciudadela Session 11: Closing keynote Chair: Gloria Gonzalez-Asequinolaza, <i>University of Navarra, Pamplona</i>; Ander Izeta, <i>Biodonostia, San Sebastian</i> 09:00 Juan Bueren, <i>CIEMAT, Madrid</i> INV17 Gene Therapy in Fanconi Anemia and LAD-I diseases: Two models of stem and mature cell diseases 19:45 Awards</p>	
<p>20:30 Networking evening</p>	

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