

# ESGCT SPRING SCHOOL

BUDAPEST | 16-19 APRIL 2024

## TUESDAY 16 APRIL

15:00-16:00	Registration
16:00-18:30	<p><b>Workshops</b> (40 students per workshop)</p> <p><b>Workshop 1: Connecting Science and Society: Communicating your work beyond the lab</b> Anna Couturier, <i>EuroGCT</i></p> <p><b>Workshop 2: How to write a scientific manuscript</b> Thomas Gallagher, <i>Human Gene Therapy</i></p> <p><b>Workshop 3: How to judge a scientific publication</b> Hildegard Büning, <i>Hannover Medical School</i></p>
19:30	<p><b>Meet the expert dinner @ Araz Restaurant, <a href="#">Continental Hotel</a></b></p> <p>H-1074 Budapest, Dohány utca 42-44.</p>

## WEDNESDAY 17 APRIL

08:30-09:00	<p><b>Welcome Address</b></p> <p><b>Juan Bueren</b>, <i>President, ESGCT</i></p> <p><b>Zoltan Ivics</b>, <i>Spring School Host and co-organiser; President elect DG-GT</i></p> <p><b>Hildegard Büning</b>, <i>ESGCT Education &amp; Training</i></p>
09:00-10:00	<p><b>Keynote</b></p> <p><b>INV01: Juan Bueren</b>, <i>Ciemat Madrid</i> Moving from concept to clinical reality</p>
10:00-11:30	<p><b>Session 1: Gene therapy tools - insights by experts (viral vectors)</b></p> <p><b>INV02: Axel Schambach</b>, <i>Hannover Medical School</i> What you should know about retro/lentiviral vector design</p> <p><b>INV03: Els Verhoeven</b>, <i>University of Nice</i> How to improve nature I – the next generation of lentiviral vectors</p> <p><b>INV04: Jacob Giehm Mikkelsen</b>, <i>Aarhus, Denmark</i> Engineered lentivirus-derived particles for delivery of gene editing tool kits</p>
11:30-12:00	<b>Coffee Break</b>

<b>12:00-13:00</b>	<b>Keynote</b> <b>INV05: Fulvio Mavilio</b> , <i>Orchard Therapeutics</i> Hematopoietic stem cell gene therapy for genetic diseases
<b>13:00-14:00</b>	<b>Lunch</b>
<b>14:00-16:00</b>	<b>Session 2: Gene therapy tools - insights by experts (viral vectors)</b> <b>INV06: Hildegard Büning</b> , <i>Hannover Medical School</i> Almost a virus – the adeno-associated virus (AAV) vector system vector design <b>INV07: Alberto Auricchio</b> , <i>Tigem Naples</i> ExpEditing AAV gene therapy <b>INV08: Luk Vandenberghe</b> , <i>Harvard Medical School</i> AAV as a vaccine platform
<b>16:00-17:00</b>	<b>Keynote</b> <b>INV09: Gloria Gonzalez-Aseguinolaza</b> , <i>Fima, Pamplona</i> AAV for in vivo gene editing to treat rare liver diseases
<b>17:00-17:30</b>	<b>Coffee Break</b>
<b>17:30-19:30</b>	<b>Session 3: Gene therapy tools - insights by experts (non-viral vectors)</b> <b>INV10: Tristan Montier</b> , <i>University of Brest</i> How to fine tune nanoparticles for aerosol delivery <b>INV11: Raymond Schiffelers</b> , <i>UMC Utrecht</i> Lipid nanoparticles and their applications in vaccination and beyond <b>INV12: Zoltan Ivics</b> , <i>Paul Ehrlich Institute, Langen</i> Wide awake and ready to move: non-viral genome engineering with Sleeping Beauty transposon vectors
<b>19:45</b>	<b>Dinner at Ruin Bar Extra with DJ and surprise</b>

## THURSDAY 18 APRIL

<b>09:00-10:00</b>	<b>Keynote</b> <b>INV13: Toni Cathomen</b> , <i>University Hospital, Freiburg</i> Revelations in Precision: Learning from on- & off-target effects of gene editing tools
<b>10:00-10:30</b>	<b>Coffee Break</b>

<b>10:30-12:30</b>	<b>Session 4: Genome and epigenome editing</b>  <b>INV14: Paula Rio</b> , <i>Ciemat Madrid</i> Gene editing with CRISPR and beyond  <b>INV15: Claudio Mussolino</b> , <i>University of Freiburg</i> Multiplexed epigenome editing in CAR T cells  <b>INV16: Raul Torres</b> , <i>Ciemat, Madrid</i> Prime editing, base editing etc
<b>12:30-13:30</b>	<b>Lunch</b>
<b>13:30-15:30</b>	<b>Session 5: iPSC technology</b>  <b>INV17: Agota Apati</b> , <i>Research Centre for Natural Sciences, Budapest</i> Advancements in induced pluripotent stem (iPS) technology and two-dimensional disease models  <b>INV18: Kornelia Szebenyi</b> , <i>Research Centre for Natural Sciences, Budapest</i> Human induced pluripotent stem cell-derived organoids for disease modelling  <b>INV19: Andras Nagy</b> , <i>Lunenfeld-Tanenbaum Research Institute, Toronto</i> Therapeutic applications
<b>15:30 -16:30</b>	<b>Keynote</b>  <b>INV20: Nathalie Cartier</b> , <i>AskBio, Paris</i> Gene therapy for CNS diseases : strategies, hurdles, results
<b>16:30-17:00</b>	<b>Coffee Break</b>
<b>17:00-17:40</b>	<b>Session 6: Fighting cancer with novel weapons</b>  <b>INV21: Hinrich Abken</b> , <i>University of Regensburg</i> CARs, TRUCKs and what is next? The evolution of CAR T cell therapy
<b>17:40 -18:40</b>	<b>Keynote</b>  <b>INV22: Vincenzo Cerullo</b> , <i>University of Helsinki</i> Dressing viruses in tumor's clothing – new strategies in anti-tumor therapy
<b>19:00</b>	<b>Boat trip on the Danube with dinner and DJ</b>

## FRIDAY 19 APRIL

<b>09:00-10:00</b>	<b>Keynote</b> <b>INV23: Anne Galy</b> , <i>Inserm ART-TG laboratory</i> Immune system: friend or foe in gene therapy
<b>10:00-10:30</b>	<b>Coffee Break</b>
<b>10:30-12:30</b>	<b>Session 7: Pre-clinical and clinical gene therapy</b> <b>INV24: Giuliana Ferrari</b> , <i>SR Tiget, Milan</i> Gene therapy for hemoglobinopathies: past, present and future <b>INV25: Federico Mingozi</b> , <i>Spark Therapeutics</i> Translating in vivo gene therapies from bench to bedside <b>INV26: Fatima Bosch</b> , <i>UAB, Barcelona</i> A new horizon in gene therapy: Moving from the treatment of rare to highly prevalent diseases
<b>12:30-13:30</b>	<b>Keynote</b> <b>INV27: Claire Booth</b> , <i>UCL London</i> It is all about accessibility
<b>13:30</b>	<b>Closing</b> <b>Alberto Auricchio, Hildegard Büning, Zoltan Ivics</b>