



## Precision Medicine and Functional Genomics 2023 – Post-Conference Workshop

**Course Title: Genome Editing: Basic Learning and Advanced Techniques**

**Duration: Two days - 15<sup>th</sup> and 16<sup>th</sup> of November 2023**

**Course Duration: 2 days**

Co-organized by the European COST action ‘Genome Editing to Treat Human Diseases’ (GenE-HumDi; action CA21113 ), an EU-funded network that connects researchers and innovators across Europe and beyond.



### PROGRAM AGENDA

DAY 1: 15 <sup>th</sup> November 2023 – Morning Session	
Time	
7:30 – 8:00	Registration
8:00 -8:10	<p>Welcome Message</p> <p>TBD</p>
8:10 – 8:25	<p>Introduction: Objectives of the Workshop</p> <p>Cristina Maccalli (Sidra Medicine) and Karim Benabdel Lah El Khlanji - (GENYO-Spain)</p>
8:25-10:25	

## Introduction: Objectives of the Workshop

### **Session 1. Gene editing techniques: advances, pros and cons**

8:25 – 9:25	Mario Amendola (INSERM-Genethon, France)	<ul style="list-style-type: none"><li>• Introduction to gene editing techniques (e.g., CRISPR-Cas9, TALENs)</li><li>• Principles of guide RNA (gRNA) design and target selection</li><li>• Assessment of gene editing efficiency and genotoxicity</li></ul>
9:25 – 10:25	Yonglun Luo (Aarhus University, Denmark)	<ul style="list-style-type: none"><li>• Design of gene editing experiments</li><li>• Sanger sequencing of gene edited samples.</li><li>• Epigenetic of gene editing</li><li>• In vivo gene editing</li></ul>
10:25 – 11:00	Coffee break	
11:00-13:00		
<b><u>Session 2. Gene editing: Clinical and Biomedical Applications</u></b>		
11:00- 11:40	Karim Benabdel Lah El Khlanji (GENYO-Sapin)	<ul style="list-style-type: none"><li>• CRISPR-Cas9 gene editing in cancer immunotherapy</li></ul>
11:40-12:20	Mario Amendola (INSERM-Genethon, France)	<ul style="list-style-type: none"><li>• CRISPR-Cas9 gene editing for Sickle Cell Anemia and <math>\beta</math>-Thalassemia</li></ul>
12:20:13:00	Yonglun Luo (Aarhus University, Denmark)	<ul style="list-style-type: none"><li>• CRISPR to decipher the mechanisms of human diseases</li></ul>
13:00-14:30	Lunch Break	

## Afternoon Session: Practical Training

### **Session 3. Introduction to Technical Platforms for Gene Editing**

Time		
14:30-14:45	Cristina Maccalli (Sidra Medicine)	<ul style="list-style-type: none"><li>• Introduction to the practical training</li></ul>

14:45-16:00	Yonglun Luo (Aarhus University, Denmark)	<ul style="list-style-type: none"> <li>Case report: exercise of design and development of gene editing with CRISPR/Cas9</li> </ul>
16:00-17:00	<p>Yonglun Luo (Aarhus University, Denmark) and Mario Amendola (INSERM-Genethon, Franc)</p> <p>Amendola (INSERM-Genethon, France)</p>	<ul style="list-style-type: none"> <li>Usage of nucleofector for Gene editing</li> <li>Introduction to nucleofection and its advantages</li> </ul>
<b>End of Day 1</b>		

<b>DAY 2: 16<sup>th</sup> November 2023</b> Location: Research Branch Floors (OPC 5 <sup>th</sup> Floor , 401A/401B)		
Time		
8:30-8:45	Cristina Maccalli (Sidra Medicine)	<ul style="list-style-type: none"> <li>Presentation of the agenda of the day</li> </ul>
8:45 – 9:45	Yonglun Luo (Aarhus University, Denmark, Mario Amendola (INSERM-Genethon, France) and Karim Benabdel Lah El Khlanji (GENYO-Sapin)	<ul style="list-style-type: none"> <li>Discussion of the case report from Day 1</li> </ul>
9:45 – 10:00	Coffee Break	
10:00 – 12:30	Sara Tomei (Integrated Genomic Laboratory)	

	<ul style="list-style-type: none"> <li>• High resolution melting, sanger sequencing and NGS platforms</li> </ul> <p>Sahar Da'as (Zebrafish Facility)</p> <ul style="list-style-type: none"> <li>• Examples of functional validation of CRISPR/Cas9 gene editing in <i>in vivo</i> zebrafish model</li> </ul> <p>Giusy Gentilcore (Precise Cellular and Molecular Phenotyping Unit)</p> <ul style="list-style-type: none"> <li>• Examples of the usage of flow cytometry for assessment and QC of gene editing</li> </ul>
12:30 – 13:30	Light Lunch Break
13:30 – 15:00	<p>Abbirami Sathappan (Advanced Imaging Laboratory)</p> <ul style="list-style-type: none"> <li>• Multiparametric analyses, live imaging and data analyses.</li> </ul> <p>Shana Jacob (Metabolomic facility)</p> <ul style="list-style-type: none"> <li>• Functional Studies through the Metabolomics Facility: principles and presentation of the platforms</li> </ul> <p>Chiara Cugno (Advanced Cell Therapy Core)</p> <ul style="list-style-type: none"> <li>• The GMP facility and the regulatory framework</li> </ul>
15:00 – 16:00	Wrap up of the training, Q&A and Conclusions
<b>End of Workshop</b>	