Comprehensive understanding of the latest advancement in CRISPR based gene editing delivery methods

Duration: Three days; 26–28 of June 2024

National Institute of Chemistry Slovenia

Ljubljana, Slovenia

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

Wednesday, 26.6. (7.30-18)

Thursday, 27.6 (9-18)

Friday, 28.6. (10-15)

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Summary:

Led by an esteemed panel of international experts, the workshop delves into the forefront of CRISPR/Cas9 gene editing tool delivery. By exploring delivery modalities and practical applications, the workshop endeavors to facilitate the effective translation of gene editing technologies into clinical use.

Key Points:

- Introduction to the transformative capabilities of the CRISPR/Cas system in genome modification for clinical application
- Exploration of diverse in vitro delivery strategies
- Examination of viral, non-viral, delivery modalities for CRISPR/Cas systems.
- Hands-on training led by experts in lipid nanoparticle (LNP) preparation, mRNA production, and machine learning for targeted delivery.
- Laboratory sessions on LNP-mediated CRISPR/Cas gene editing, offering participants invaluable insights and practical skills.
- Discussions on the clinical applications of nanoparticles, approved gene therapies, and case reports analysis, led by international thought leaders

Trainers:

1. **Dr. Duško Lainscek (National Institute of Chemistry, Slovenia)**
2. **Dr. Dhanu Gupta (Oxford University, United Kingdom)**
3. **Dr. Claudio Mussolino (Freiburg University, Germany)**
4. **Dr. Karim Benabdellah (Fundación progreso y salud, Granada Spain)**

Local Support:

1. Špela Malenšek (National Institute of Chemistry, Slovenia)
2. Peter Pečan (National Institute of Chemistry, Slovenia)
3. Tadej Satler (National Institute of Chemistry, Slovenia)
4. Jure Bohinc (National Institute of Chemistry, Slovenia)

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Wednesday, 26.6. (7.30–18.00)

Location: Great Lecture Hall, NIC

7.30-8.00 Registration

8.00-8.15. Welcome (Karim Benabdel Lah El Khlanji, PhD; Duško Lainšček, PhD)

8.15.-8.30 Objectives of the workshop (Duško Lainšček, PhD)

Session 1: CRISPR/Cas system–a powerful tool for genome modification

8.30-9.15 CRISPR/Cas system gene editing tool-introduction (Claudio Mussolino, PhD)

9.15-10.00 CRISPR/Cas delivery in vitro and in vivo-introduction (Dhanu Gupta, PhD)

10.00-10.30 Coffee break

Session 2: Delivery modes for the CRISPR/Cas system

10.30-11.15 Exploring viruses for the CRISPR/Cas system delivery (Duško Lainšček, PhD)

11.15-12.00 Exploring IDLV and Inducible LV-Variants for Versatile Delivery Application (Karim Benabdel Lah El Khlanji, PhD)

12.00-12.45 Extracellular vesicles: a non-viral method for CRISPR delivery (Dhanu Gupta, PhD)

12.45-13.30 Lipid nanoparticles-a new method for genome editing tool delivery (Duško Lainšček, PhD)

13.30-14.30 Lunch break

Session 3: Practical approaches of LNP usage as a CRISPR tool transfer

PRACTICAL TRAINING with THEORETICAL BACKGROUND

14.30-14.45 Introduction to practical training (Duško Lainšček, PhD)

14.45-15.30 Theory in LNP preparation and subsequent characterization (Špela Malenšek, Peter Pečan)

15.30-16.15 mRNA production for genome editing tools (Claudio Mussolino, PhD)

16.15-17.00 Using Machine learning and AI for de novo binder design for cell targeted delivery (Tadej Satler)

17.00-17.30 Cas9 protein isolation-tricks and tips for protein isolation (Jure Bohinc)

17.30-18.00 Wrap up of the first day, presenting case studies for the discussions in groups for CRISPR delivery (Duško Lainšček, PhD)

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Thursday, 27.6. (9–18)

Location: Great Lecture Hall, Department of Synthetic Biology and Immunology, NIC

PRACTICAL TRAINING with THEORETICAL BACKGROUND

9.00-9.15 Presentation of the agenda for the day (Duško)

9.15-10.00 Protocol for LNP preparation (Špela, Peter)

10.00-13.00 CRISPR/Cas gene edit mediated by LNP delivery - working in laboratory (Špela Malenšek, Peter Pečan, Duško Lainšček, PhD)

13-14.30 Lunch break

14.30-17.30 CRISPR/Cas gene edit mediated by LNP delivery - working in laboratory (Špela Malenšek, Peter Pečan, Duško Lainšček, PhD)

17.30-18.00 Wrap up

Friday, 28.6. (10–15)

Location: Great Lecture Hall, NIC

10.00-10.30. Discussions about the past day; Presentation of the agenda for the day (Duško Lainšček, PhD)

10.30-11.15 Nanoparticles in clinical use (Duško Lainšček, PhD; Dhanu Gupta; PhD)

11.15-12.00 Approved gene therapies and their delivery (Claudio Mussolino; PhD)

12.00-13.00 Lunch break

13.00-15.00 Case reports discussions

15.00-15.15 Wrap up, End of the Workshop

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