DABE Pre-conference Symposium

January 14, 2017

Workshop on the use of CRISPR-Cas9 for Gene Editing: Principles and Practices

Session I. Introduction to CRISPR-Cas9 Genome Editing & Strategies for Defining and Improving Specificity

Dr. Shengdar Tsai. Department of Hematology
St. Jude Children's Hospital, Memphis, Tennessee, USA

RNA-guided CRISPR-Cas9 nucleases have transformed genome editing, due to the simplicity and robustness with which they be programmed to introduce targeted double-strand breaks (DSB) into the genomes of living cells and organisms. In this workshop session, a broad overview of the landscape of CRISPR-Cas9 genome editing will be provided, and state-of-the-art strategies for both defining and improving the genome-wide specificities of CRISPR-Cas9 nucleases will be discussed. An extensive Q&A session will enable participants to discuss best practices for applying these transformative technologies in their own labs.

Session II. Direct CRISPR-Cas9 microinjection into mammalian embryos

Dr. Lluis Montoliu. Centro Nacional de Biotecnologia (CNB-CSIC), Campus de Cantoblanco, Madrid, Spain

In this section we will be focusing on the delivery of CRISPR-Cas reagents to mammalian embryos by means of direct microinjection. The workshop will cover the different CRISPR-Cas-related molecules one can microinject, different applications including: small insertion and deletions, large deletions, inversions, duplications, and large insertions (knock-ins). This section will also include a reference to single versus multiple simultaneous genetic modifications. Finally, we will discuss the current knowledge on how to minimize non-homologus end-joining (NHEJ) and boost homology directed repair (HDR). An extensive Q&A session will enable participants to discuss best practices for applying these technologies in their own labs.

Session III. Gene editing using CRISPR-Cas9 as a therapeutic strategy

Dr. Deepak Reyon, Editas Medicine, Cambridge, Massachusetts, USA

The continual maturation of this technology supports the transition of gene editing from being a powerful laboratory tool, to becoming a viable therapeutic strategy. In this workshop session, we will explore what it takes to transition this revolutionary technology from the laboratory to the clinic. These challenges, along with various strategies, and lessons learned, will be discussed using real-world examples. The workshop will be followed by a Q&A session where participants can discuss not only the use of CRISPR-Cas9 as a therapeutic, but also as a reagent to generate crucial tools that enable drug discovery.

Poster Session. As part of the workshop there will be a poster session to showcase your work. Details to follow.

Schedule

8:15 – 8:30	Introductions to workshop, thanks to sponsors
Session I	**Introduction to CRISPR-Cas9 Genome Editing and Strategies for Defining and Improving Specificity**
8:30 – 9:30	Session I Part 1
9:30 – 10:30	Session I Part 2
10:30 –11:00	Coffee break
Session II	**Direct CRISPR-Cas9 microinjection into mammalian embryos**
11:00 – 12:00	Session II Part 1
12:00 – 1:00	Session II Part 2
1:00 – 2:30	Box Lunch and Poster Session
Session III	**Gene editing using CRISPR-Cas9 as a therapeutic strategy**
2:30 – 3:30	Session III Part 1
3:30 – 4:30	Session III Part 2
4:30 – 5:30	Poster awards and Panel Discussion
6:30 – 8:30	Social - Location and time to be determined

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