CENTRE FOR EPIDEMIOLOGY & BIOSTATISTICS

W.I.P. Seminar

FIRST-IN-HUMAN STUDY OF FEASIBILITY AND SAFETY OF CCR5-MODIFIED BLOOD STEM CELLS IN HIV-1 INFECTED SUBJECTS

Rodica Stan, PhD
Centre for Gene Therapy, City of Hope, Duarte, California, US

We developed a novel therapeutic strategy for HIV-1 infection by engineering HIV-resistant immune cells via gene editing of autologous hematopoietic stem/progenitor cells (HSPC). A Zinc Finger Nuclease (ZFN) mRNA construct was designed to selectively disrupt the chemokine receptor 5 (CCR5), a co-factor required for HIV-1 infection of human cells, in HSPC. Thus far, we manufactured and infused eight ZFN-CCR5-modified HSPC products as autologous "transplants" after busulfan conditioning. Preliminary results are now available from the ongoing safety/feasibility clinical trial (NCT02500849). This is the first-in-human use of ZFN genome editing of HSPC, and preliminary data show that conditioning with busulfan and infusion of autologous CCR5-ZFN-modified HSPC are safe and well tolerated in HIV-infected subjects.

Dr Stan works in the Centre for Gene Therapy, Hematologic Malignancies and Stem Cell Transplantation Institute at City of Hope in Duarte, California, US. She has a research interest in translational medicine, with a major commitment to contributing to the development of new therapies, particularly in the area of gene therapy for HIV/AIDS. She had a substantial input to the preparation and approval of the Investigational New Drug applications for the first lentivirus-based and Zinc Finger Nuclease-based stem cell gene therapy projects in HIV/AIDS using blood/hematopoietic stem cell transplantation in AIDS lymphoma and HIV-infected patients, respectively. Dr Stan has vast regulatory and project management experience from the previous positions she held in the US and Australia.

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10:30 – 11:30am, Monday 25th June 2018
Seminar Room 515, Level 5, 207 Bouverie St
Carlton Vic 3053

All welcome RSVP not required