



Doctorate program
Milan
EXPERIMENTAL
MEDICINE



UNIVERSITÀ
DEGLI STUDI
DI MILANO

International PhD Program in Experimental Medicine

D-MEM SEMINAR

MARIA ESTER BERNARDO

Università Vita-Salute San Raffaele, Milano
San Raffaele Telethon Institute for Gene Therapy

April 5, 2023 - 4.00 pm

Aula C, LITA Segrate

HAEMATOPOIETIC STEM/PROGENITOR CELL-GENE THERAPY IN INBORN ERRORS OF METABOLISM

Over the last 30 years, allogeneic haematopoietic stem cell transplantation (allo-HSCT) has been adopted as a therapeutic strategy for many inborn errors of metabolism (IEM), due to the ability of donor-derived cells to provide life-long enzyme delivery to deficient tissues and organs. However, patients are left with a substantial residual disease burden and allo-HSCT is still associated with significant morbidity and mortality. Haematopoietic stem/progenitor cell gene therapy (HSPC-GT) has been established in the 1990s for the treatment of selected monogenic primary immunodeficiencies and over the past few years, its use has been extended to a number of IEM. HSPC-GT is particularly attractive in neuro-degenerative IEM, as gene corrected haematopoietic progenitors can deliver supra-physiological enzyme levels to difficult-to-reach areas, such as the brain and the skeleton, with potential increased clinical benefit. The number of clinical trials in the IEM field is rapidly increasing and some HSPC-GT products recently received market approval. This presentation will focus on the development of ex vivo HSPC-GT in IEM, looking at recent results from gene therapy clinical trials and highlighting future perspective in this field.

Scan this QR code or
click [HERE](#) to join the seminar online on MS Teams



facebook

PhD in Experimental Medicine - University of Milan

twitter

@DMEM_PhDunimi